



제넥신-툴젠 통합법인

2019. 7. 1

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Why & Why Now

ToolGen's Technology & IP

Genexine Business Development

ToolGenexine Development Strategy

Operation Strategy

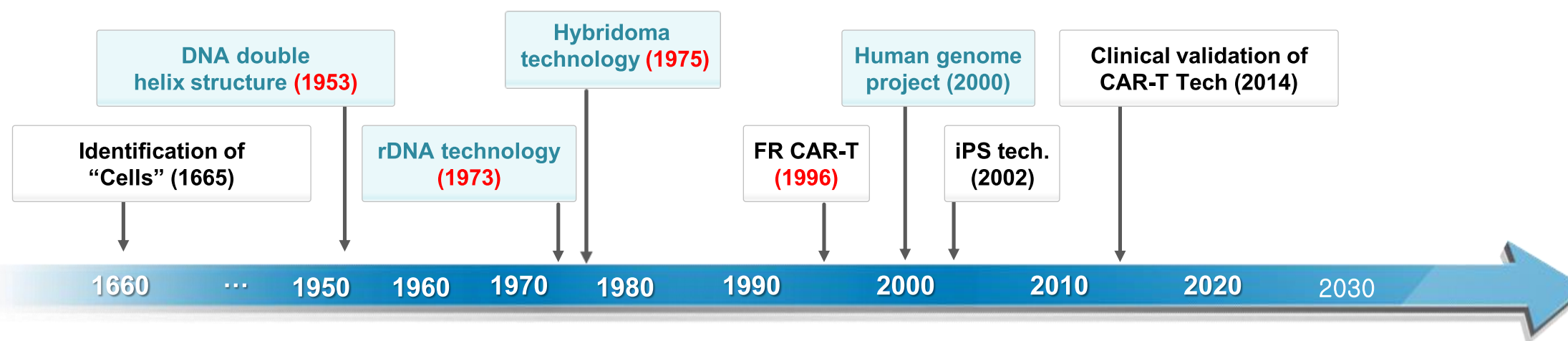
Why & Why Now

유전자 치료제 시대를 선점

“Gene and cell therapy, forecasted to go through ‘inflection point’ to grow into mainstream therapeutic modalities in 5 years time” (expected CAGR 58% to 2024)

April 2019, McKinsey & Company

Paradigm Shift in Innovative Biologics



1st wave

r-Protein

- Insulin (Genentech, '82)
- EPO (AmGen, '89)

Long-acting protein

2nd wave

Monoclonal Ab

- Rituximab (Genentech/Idec, '97)
- Enbrel (AmGen, '99)

Multi-specific Ab

3rd wave

Gene Therapeutics

- Oncolytic HSV for cancer (Amgen, '15)
- HSC/retrovirus for SCID (GSK, '16)
- CAR-T for Cancer (Norvartis, '17)

*DC therapy: JW (2007)
T cell therapy: Green cross (2007)

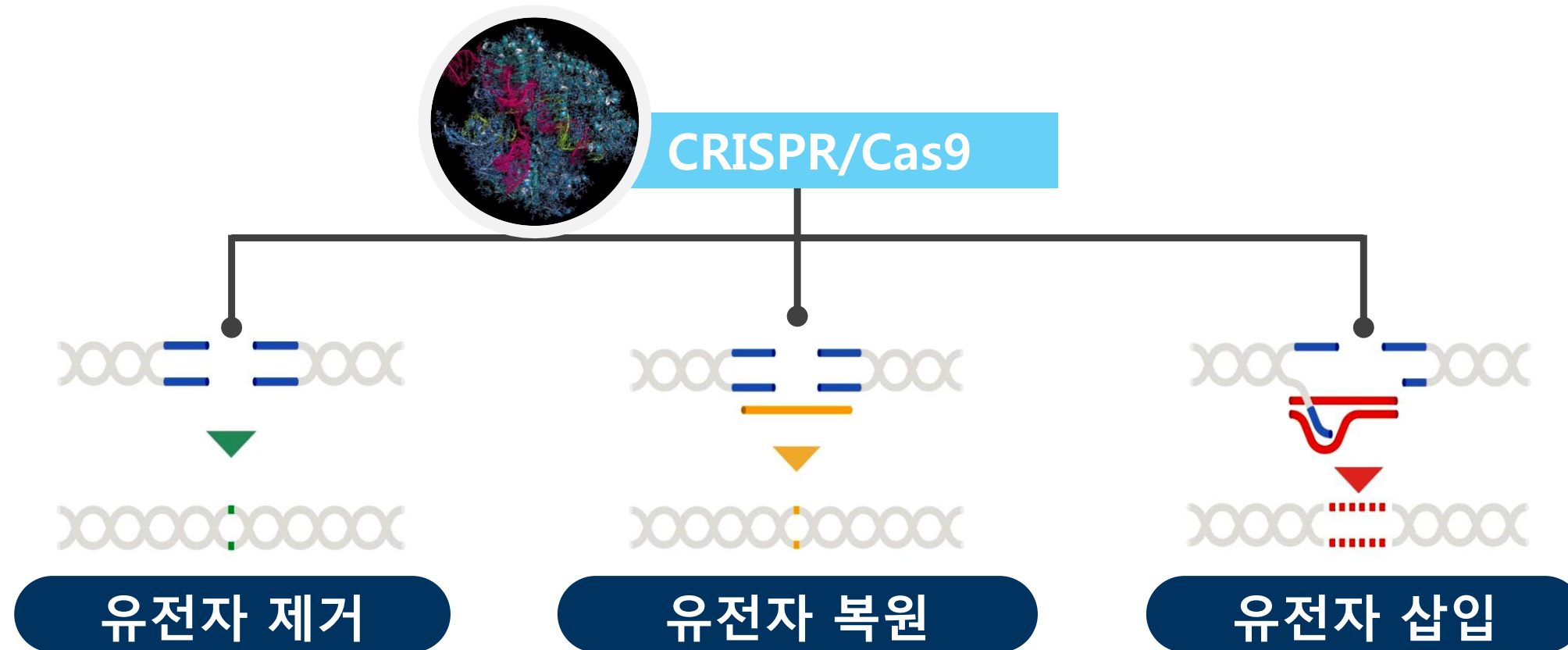
** Adenovirus/p53: Gendicine (2004)
Oncolytic virus : Rigvir (2004)
AAV/ LPLD : Uniqure (2012)

ToolGen's Technology & IP

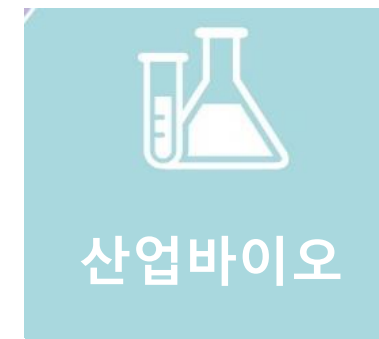
*3세대 유전자 가위 (CRISPR/Cas9) 기술은 암과 AIDS
뿐만 아니라 희귀난치병 치료나 작물, 가축개량,
미래식량(Clean meat) 분야에서 빠르게 확산되고 있다.*

CRISPR/Cas9 유전자교정 기술

- 정확하게 유전자를 교정 할 수 있는 유전자가위
- 유전자 고장에 의한 질병을 근본적으로 치료할 수 있는 최초의 혁신적 기술

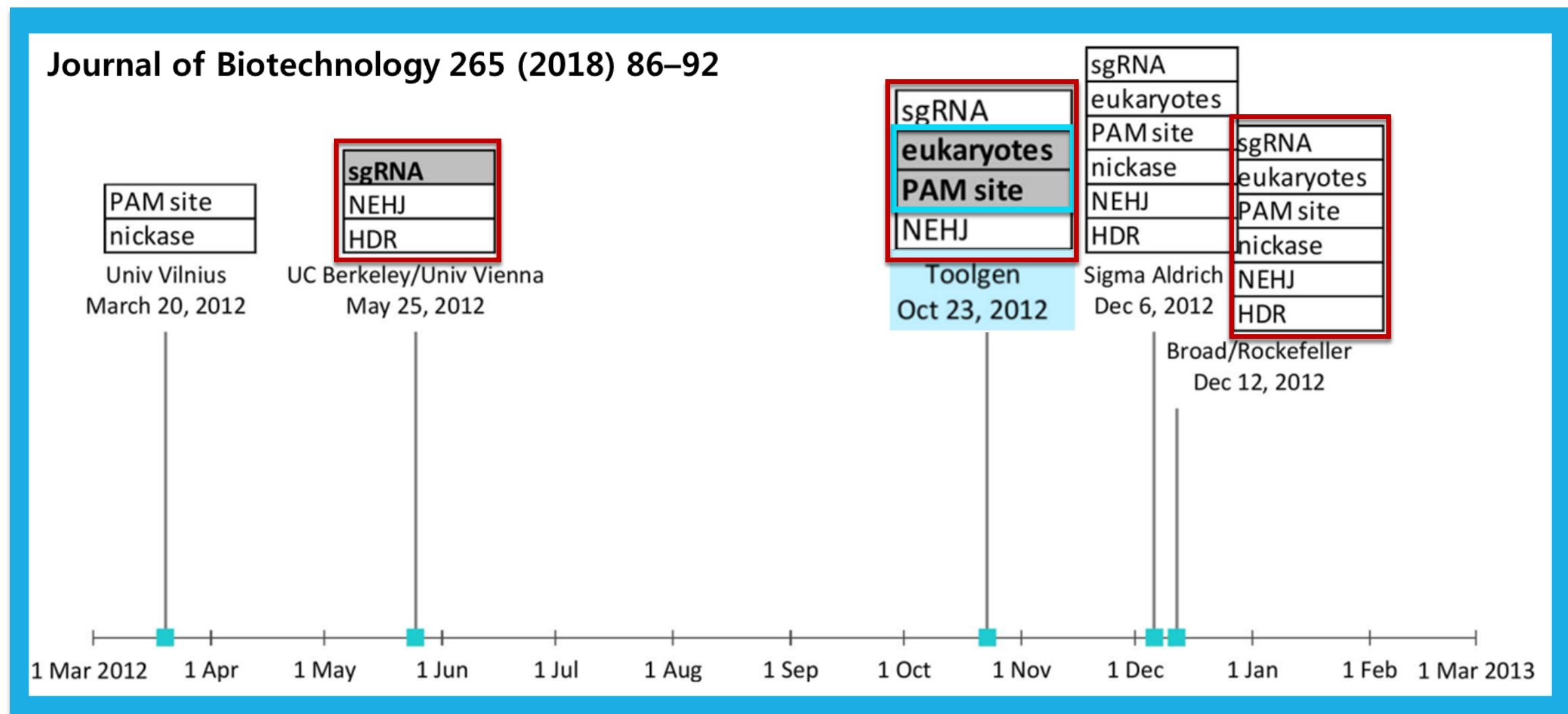


적용분야



CRISPR 유전자교정 개발 선도그룹의 특허 출원

툴젠 CRISPR/Cas9 특허 → 빠른 출원일 + 중요 발명 요소



툴젠의 CRISPR 특허 현황

Expanding Territory of Blocking Claims

PCT/KR2013/009488
(National Phase in 10 countries)

■ IP ownership

: L/O 및 유전자가위 원천기술 IP 확장

■ IP portfolio 확장

: 치료제 (Therapeutics), 동식물분야(GreenBio) 등



- ✓ US Top Law Firm "Jones Day" 을 통해 특허권 확보에 총력
- ✓ 사내에 미국 및 한국 변호사들로 구성된 특허전담팀 운용
- ✓ 기타 국내외 특허법인 및 전문가들을 통한 자문

Valuation of CRISPR-based Therapeutics Co.



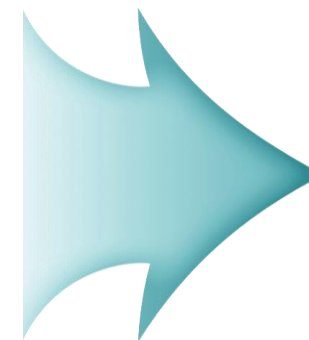
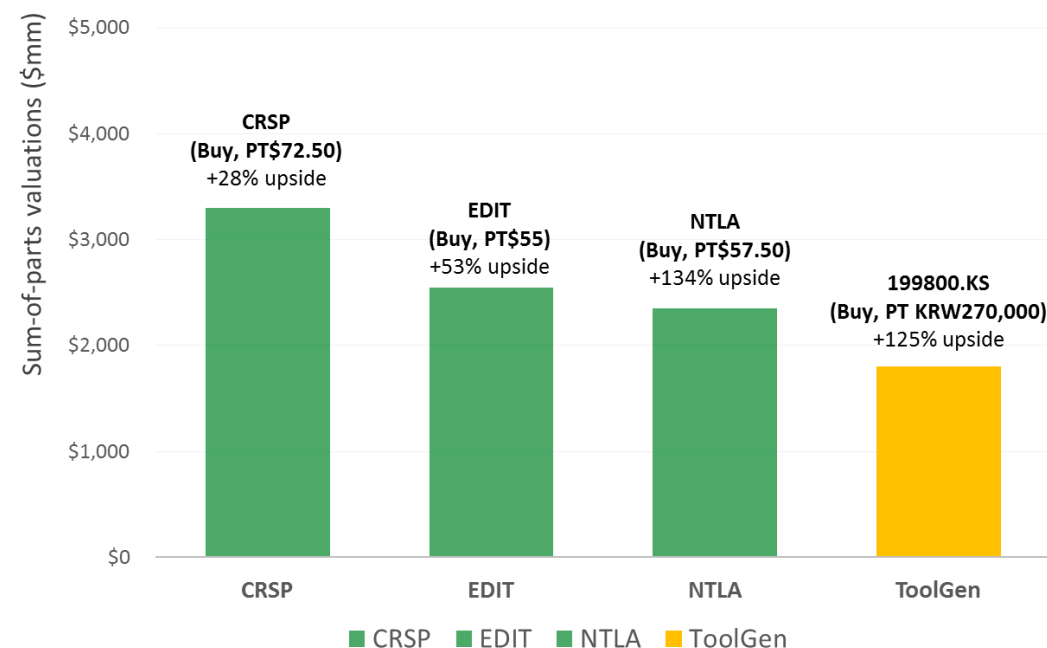
- Company: CRISPR Therapeutics
- 국가: 스위스
- 시총: 2.55B
- 임상: 임상 1/2상 3개(with Vertex) 외 전임상 8개



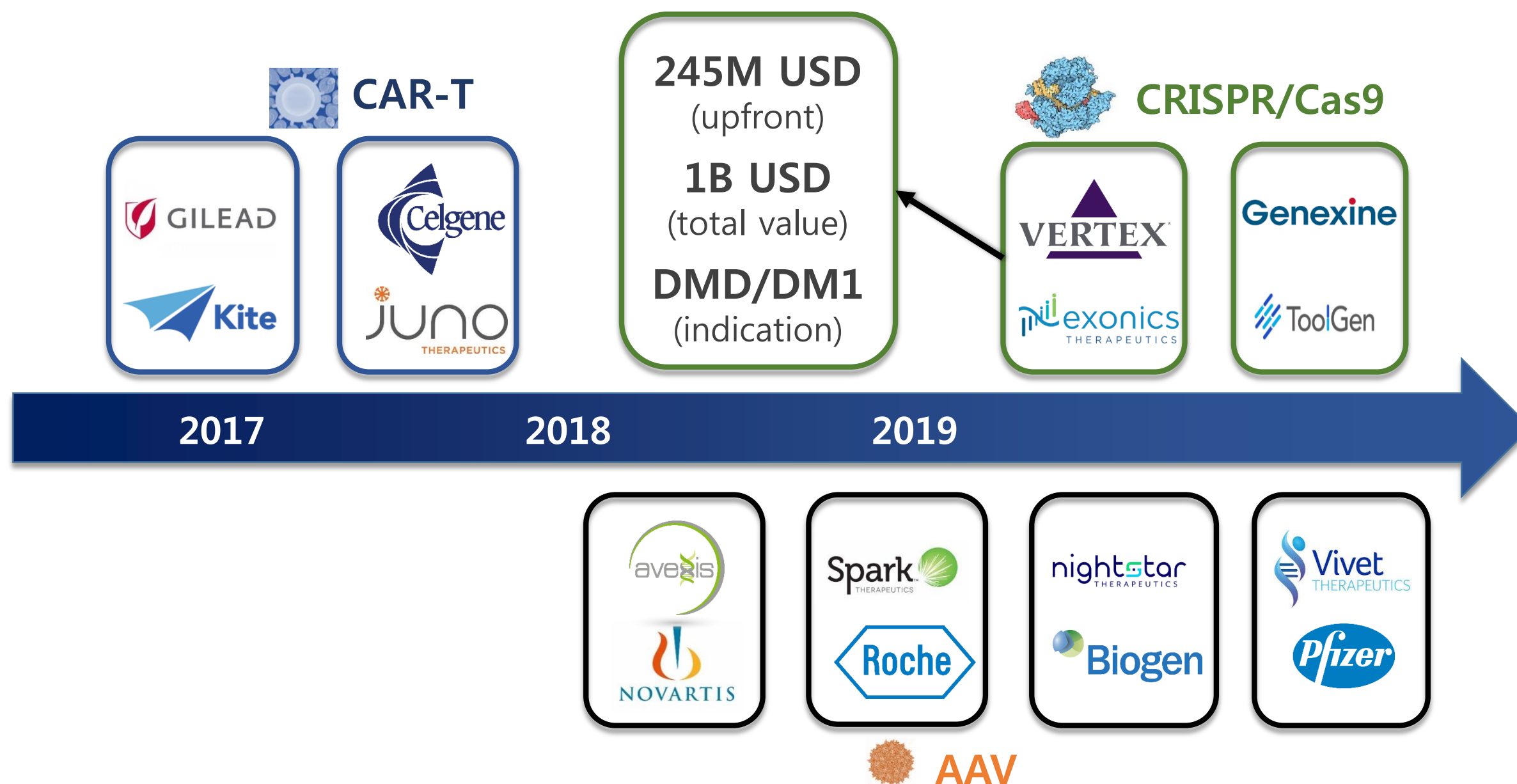
- Company: editas Medicine
- 국가: 미국
- 총: 1.13B
- 임상: 임상 1/2상 1개, Discovery 단계 7개 (Eye Diseases, Engineered T Cells 등)



- Company: Intellia Therapeutics
- 국가: 미국
- 시총: 687.49M
- 임상: Research 단계 5개, Novartis Programs 비공개



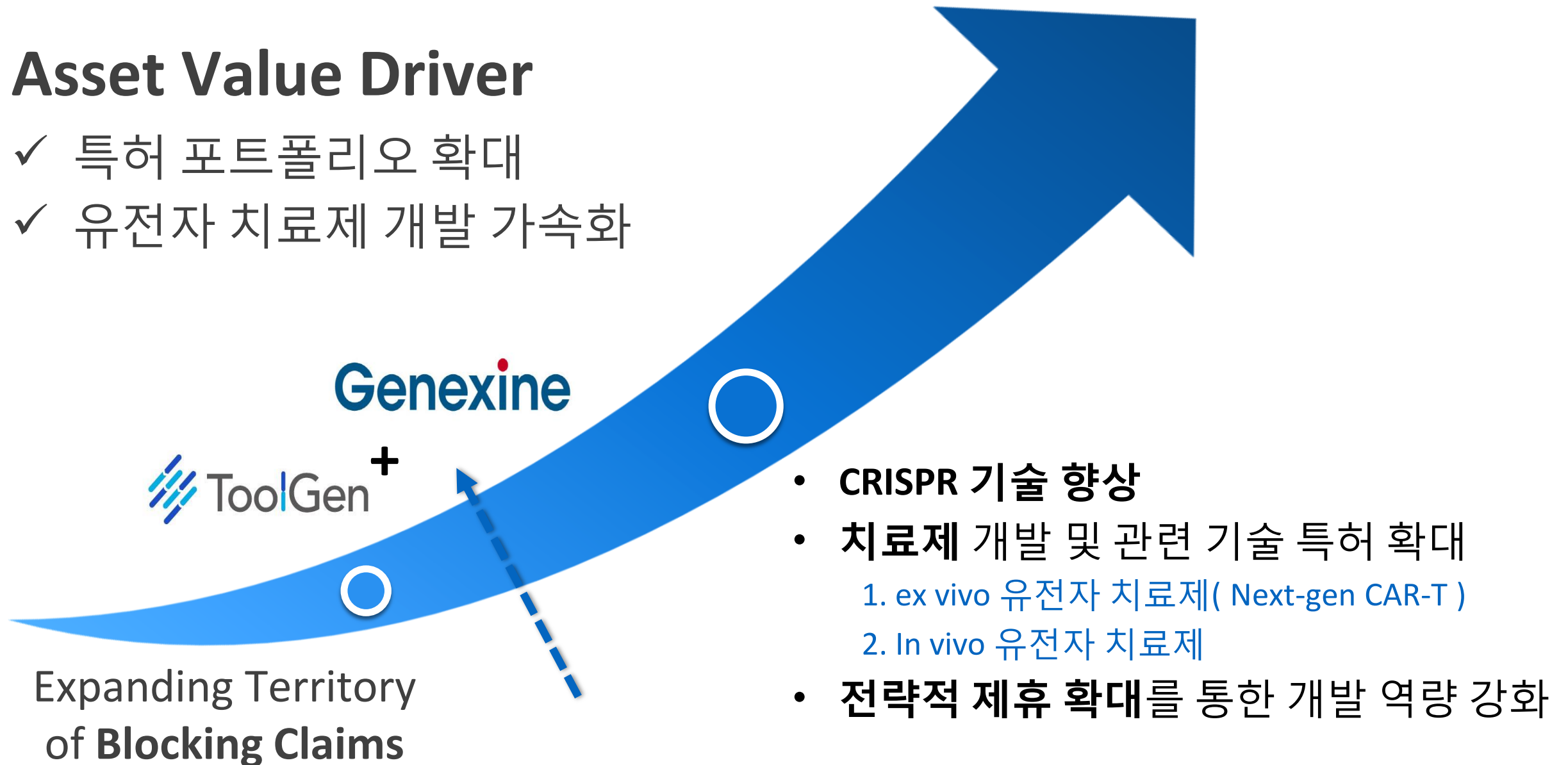
Valuation of CRISPR-based Therapeutics Co.



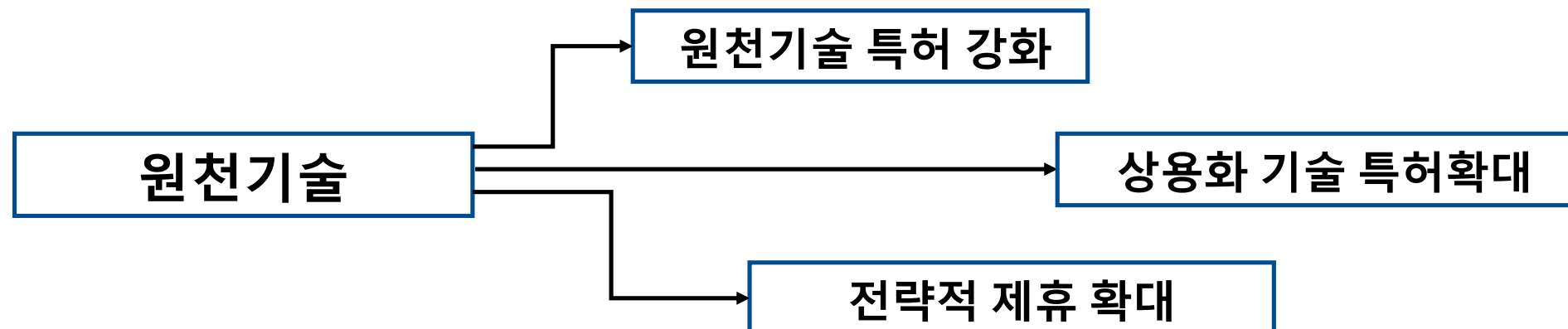
툴제넥신의 신약 개발 전략

Asset Value Driver

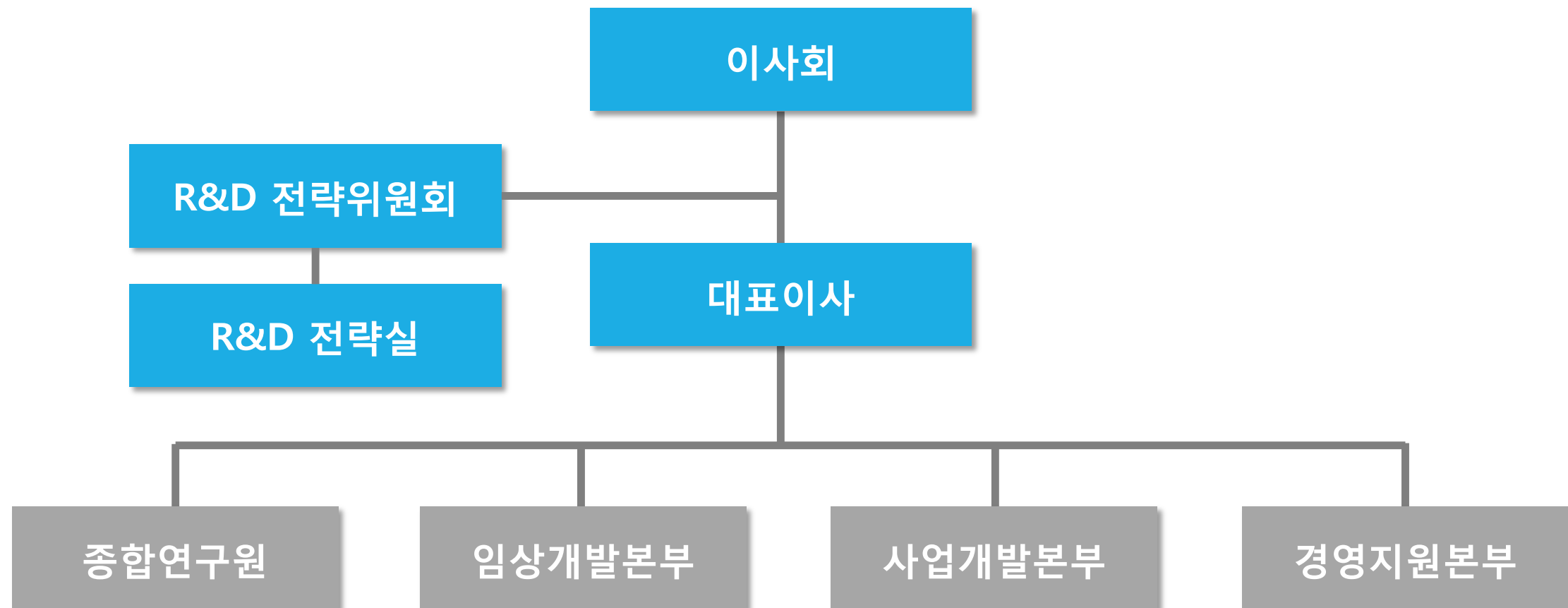
- ✓ 특허 포트폴리오 확대
- ✓ 유전자 치료제 개발 가속화



- CRISPR 기술 향상
- 치료제 개발 및 관련 기술 특허 확대
 - 1. ex vivo 유전자 치료제(Next-gen CAR-T)
 - 2. In vivo 유전자 치료제
- 전략적 제휴 확대를 통한 개발 역량 강화



통합법인 조직 운영안



■ R&D전략위원회 설치

➤양사 CTO, 연구소장 및 대표이사 등으로 구성

➤R&D전략위원회의 역할

❖미래 신기술 창출, 차세대 파이프라인 구축, 신규사업 기획

■ 통합법인의 대표이사는 기술과 경영, 사업화 경험과 역량이 뛰어난 경영인 선임 예정

■ 2021년 마곡 신 사옥 완공 후 통합 운영

ToolGenexine Outlook

Lymphopenia Drug
(hyleukin-7)

HPV+ Cancer
(GX-188, 140, 200)

- Bi- & tri-specific Ab**
- Cancer,
 - autoimmune disease
 - Metabolic disease

Genome editing

**hFc platform & Ab
Engineering**

**Know-how in CMC &
clinical development**

**Cell & gene
therapeutics**

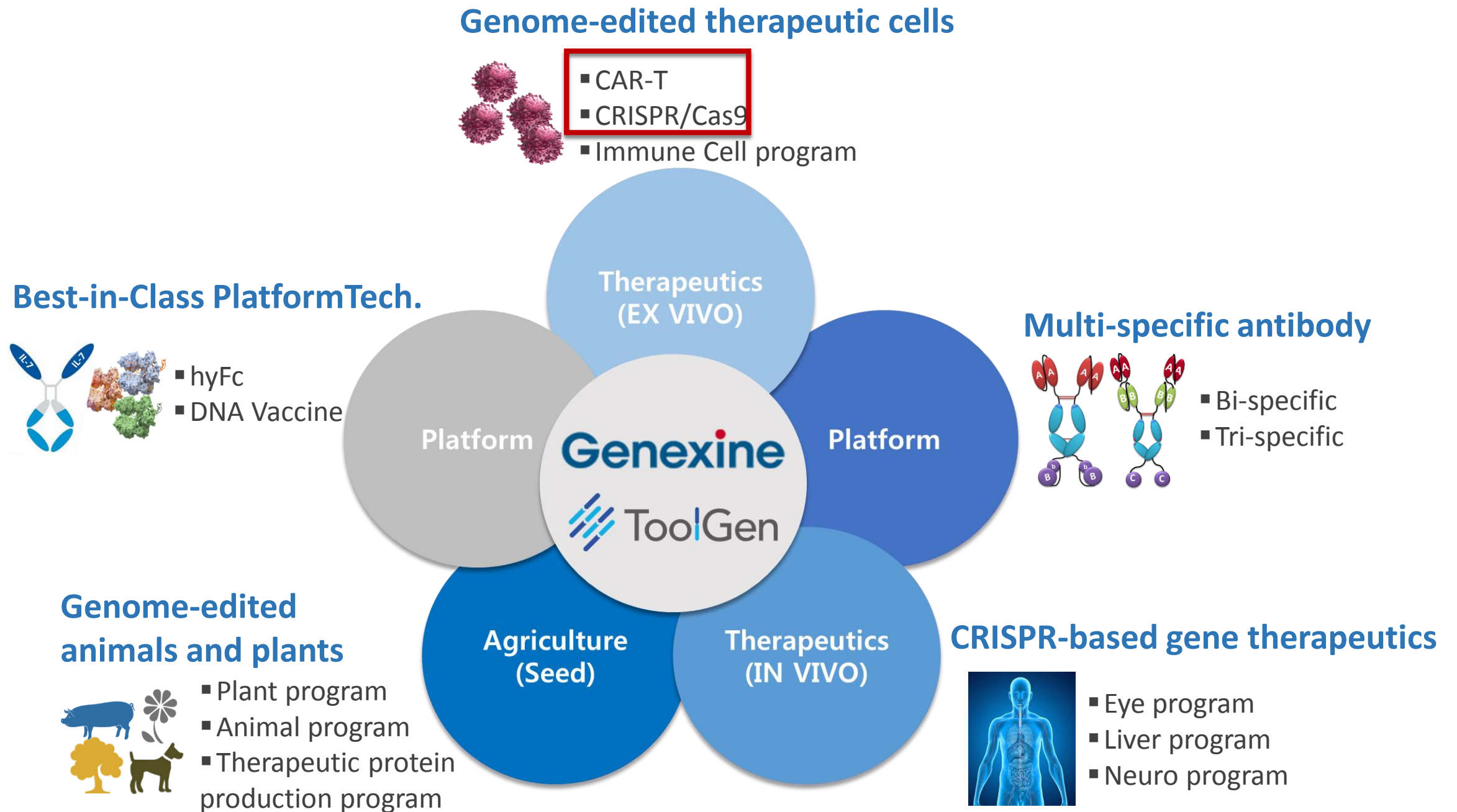
- allogeneic CAR-T
- eDC vaccine
- AIDS etc.

**In Vivo Gene
therapeutics**

- Wet AMD, CMT1A
Hemophilia B

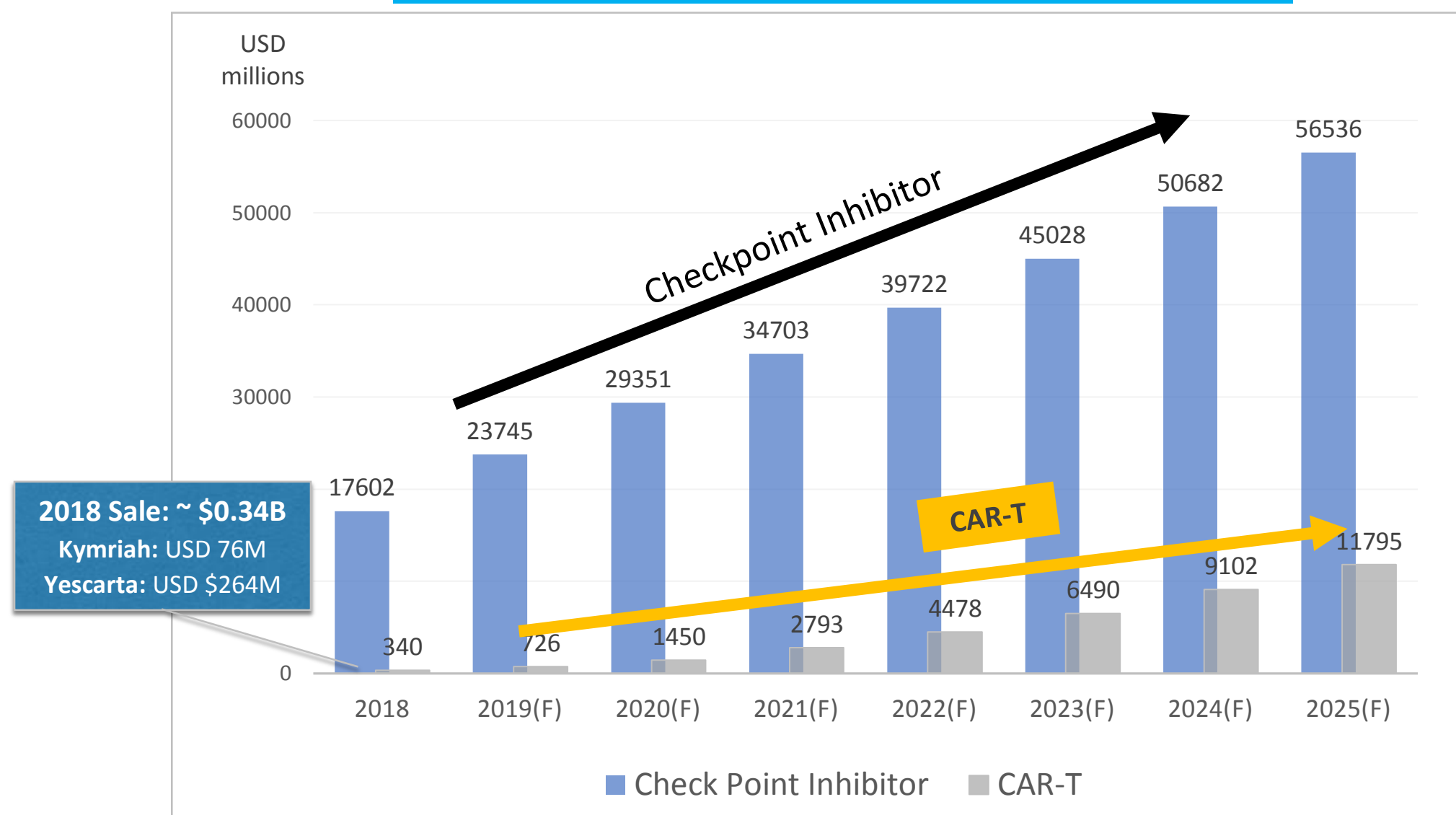
Green Bio

**Great Company Developing Innovative &
blockbuster Drugs for hopeless Patients**



Relatively Small Market : CAR-T Sales Forecast

Market Size Comparison

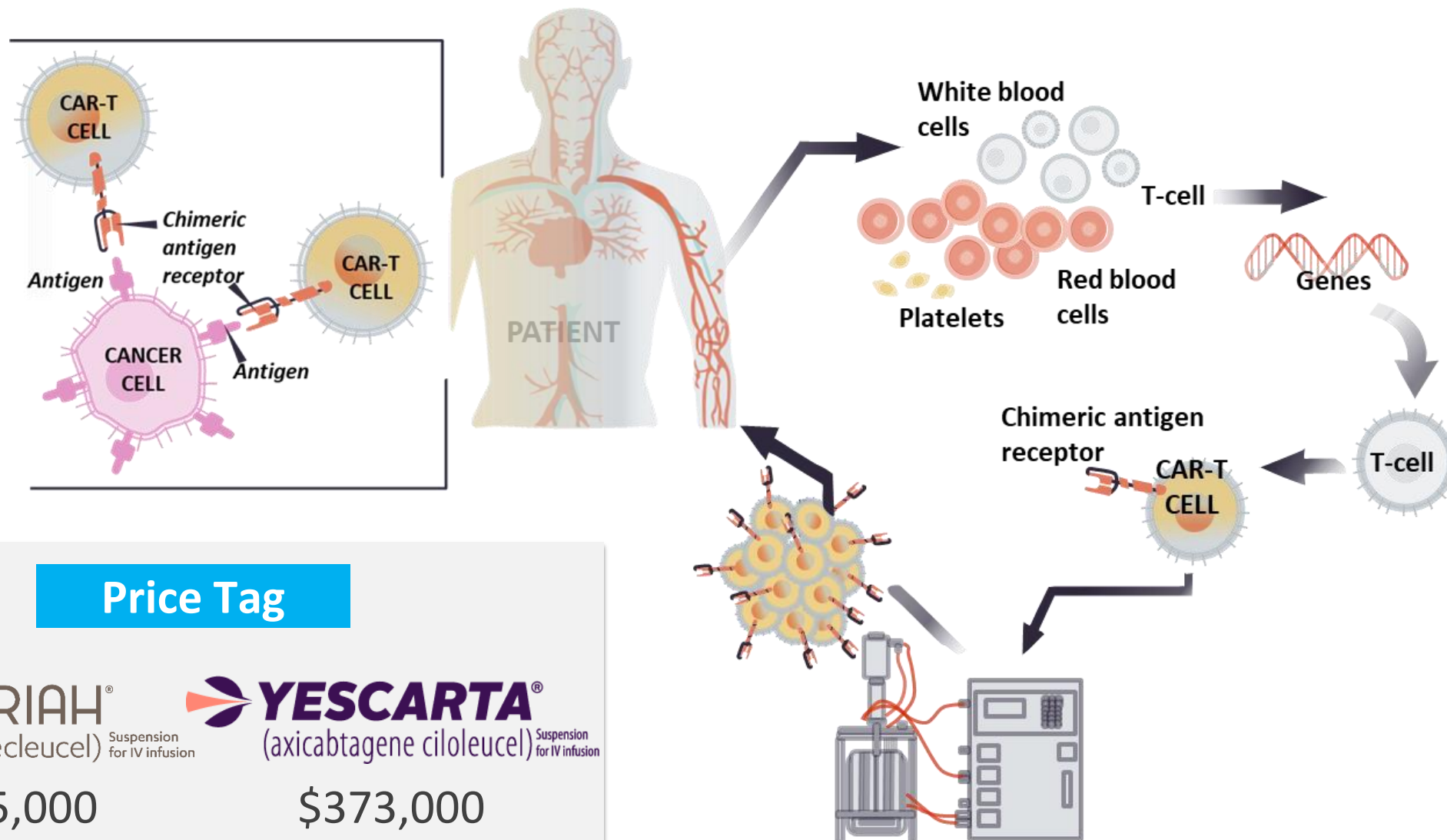


Source: GlobalData Market Intelligence

Relatively small market size compared to checkpoint inhibitor
because of **Autologous & High Price**

Current CAR-T Treatments

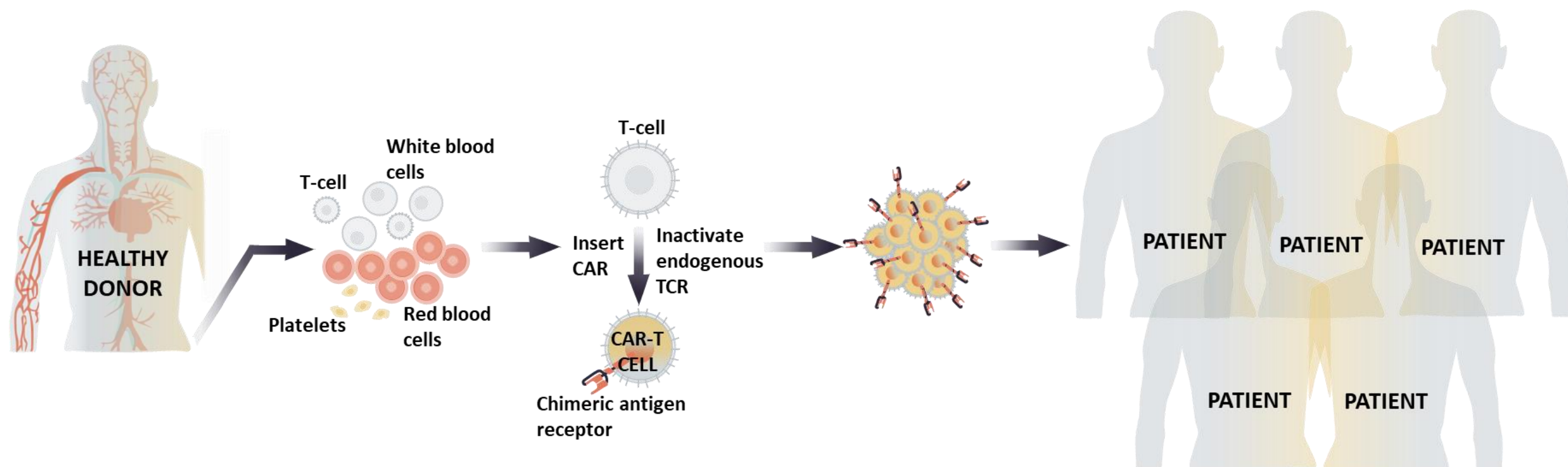
Autologous CAR-T



Autologous & High Price

Personalized to Industrialized

Autologous CAR-T → Allogeneic CAR-T



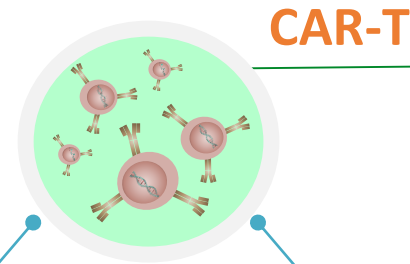
Key Features	Cell Therapy 1.0	Cell Therapy 2.0
Cell Source	Patient Cells	Master Cell Line
Manufacturing	Personalized	Off-the-Shelf
Overall Paradigm	Patient-centric	Product-centric

Use of Genome Editing in CAR-T by ToolGen

Next-generation CAR-T

기능성 향상

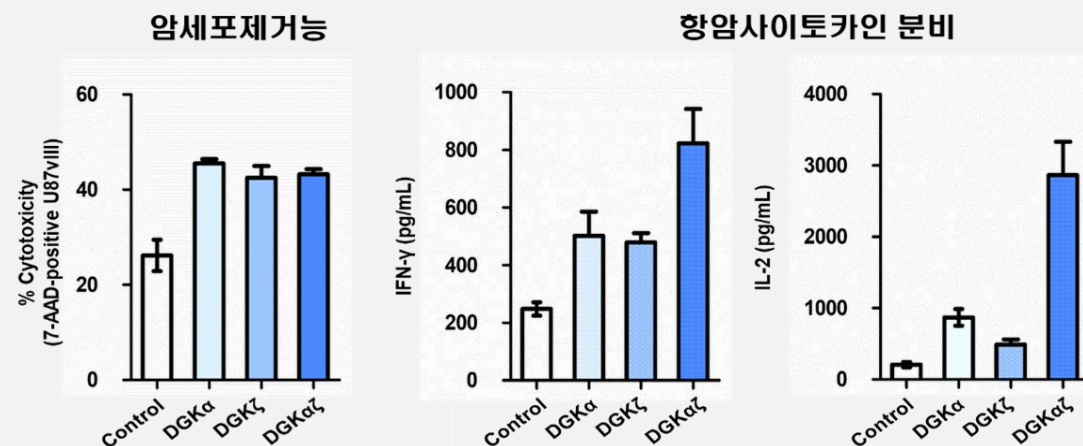
범용성 향상



향상된 CAR-T 기능 → 고형암 치료 가능성 확인

- 특허 출원, 논문 게재 (Cancer Research, 2018)

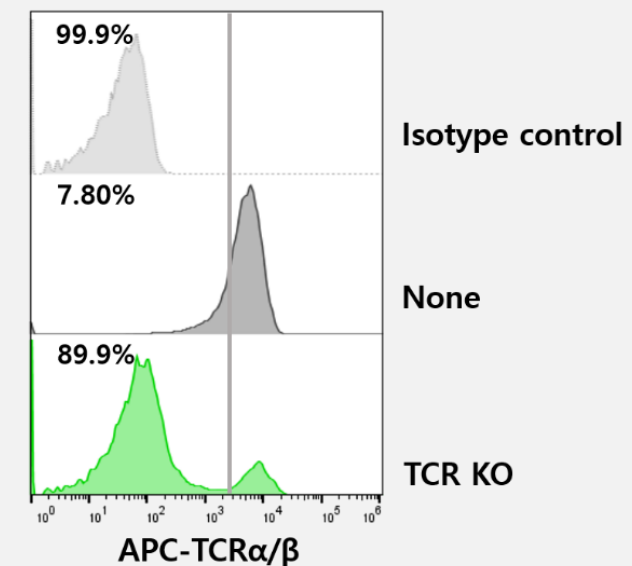
DGK KO에 의한 CAR-T 기능성 향상



범용 CAR-T를 위한 Engineering

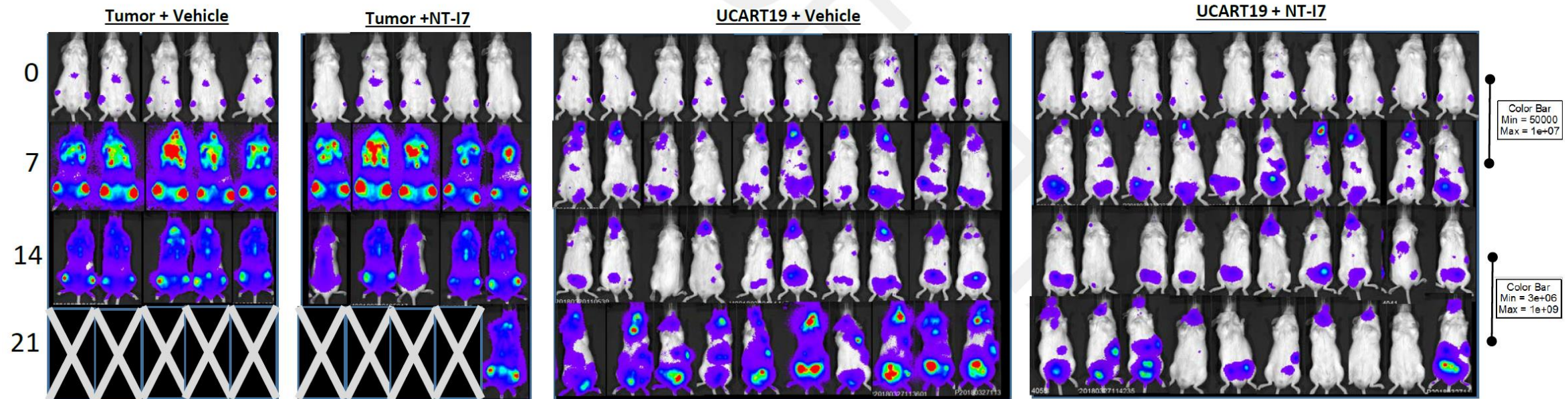
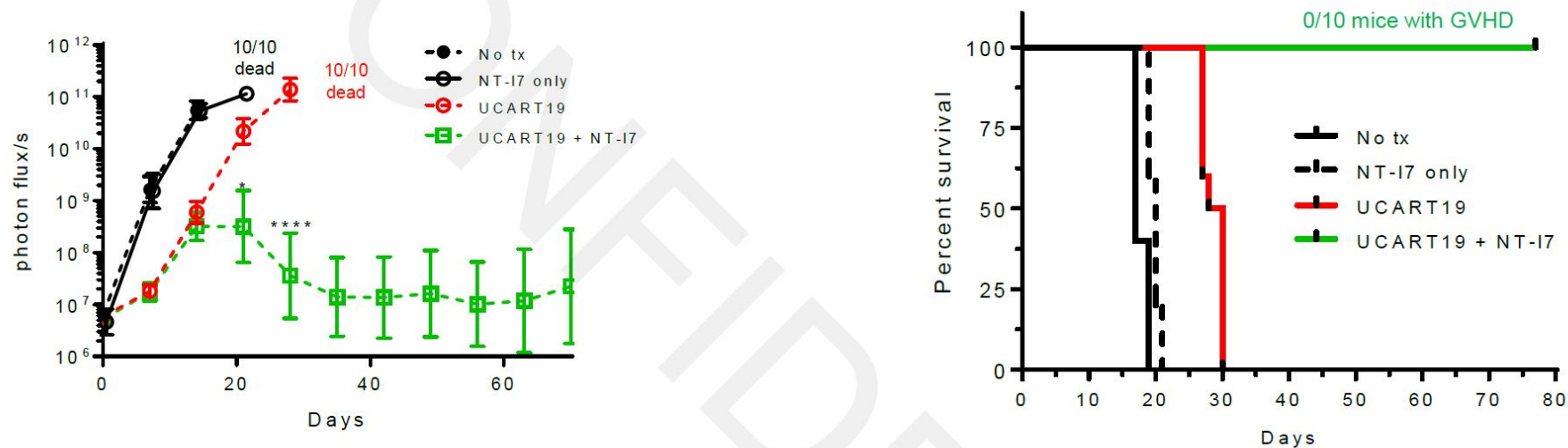
- Low cost, Short time → mass production

TCR 유전자의 효율적인 Knock out



Allogeneic CAR-T & Hyleukin-7

UCART19 and GX-I7 kill Ramos and indefinitely prolong survival



Washington University CONFIDENTIAL

준비되어 있는 CAR-T

유전자교정



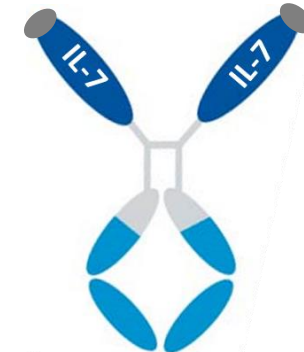
새로운 기능이 부과된 T-Cell
Gemone-edited T

美 CoImmune 지분취득



CAR-T 임상 및 사업화 기반
Manufacturing

Hyleukin-7



CAR-T 증폭 및 효능 향상
Combination Therapy

***Blockbuster
Cancer Therapeutics***

Business Strategy

신약의 가치 극대화 및 조기 수익 확보를 통한
성공 사례 창출을 최우선으로

개량 신약

상업화를 할 수 있는 초기 단계인 임상 3상 초기에 글로벌 L/O

혁신 신약

환자에서 효능 입증하는 임상 2상 단계에서 글로벌 L/O

개량 신약

Late Stage (Phase 2b-3)

GX-E2 (EPO-hyFc)

GX-G3 (G-CSF-hyFc)

GX-H9 (hGH-hyFc)

Early Stage (Phase 1- 2a)

GX-G6 (GLP-1-hyFc)

GX-G8 (GLP-2-hyFc)

혁신 신약

Early Stage (Phase 1- 2a)

HyLeukin-7 (IL-7-hyFc)
(Immuno-Oncology)

GX-188 (GX-200, -140)*
(자궁 경부전암/암 DNA Vaccine)











* 2nd Generation Pipeline

Preclinical Stage

Multi-Target Antibody Drugs
(Immuno-Oncology)

PD-L1-hyFc
(Autoimmune Diseases)

Toolgen Pipelines in Development

Program	Editing Type	Delivery	Discovery	Preclinical	Goal of 2019
In vivo: Eye					
Age-related Macular Degeneration	Knockout (NHEJ)	AAV		2019(F)	Preclinical study
Diabetic Retinopathy	Knockout (NHEJ)	AAV			Scientific POC
Genetic and infectious Diseases	Correction (HDR) / Knockout (NHEJ)	AAV			Therapeutic editing strategy establishment.
In vivo: CNS/PNS					
Charcot-Marie-Tooth Disease	Knockout (NHEJ)	AAV/LNP		2020(F)	Therapeutic POC
Huntington Disease	Knockout (NHEJ)	AAV/LNP			Therapeutic editing strategy establishment.
In vivo: Liver					
Liver Biofactory Platform (Hemophilia B)	Insertion (HDR)	AAV/LNP		2021(F)	Therapeutic POC
Hemophilia A	Inversion (NHEJ)	AAV			Scientific POC
HBV	Knockout (NHEJ)	AAV/LNP			Therapeutic editing strategy establishment.
Ex vivo: CAR-T					
STYX-T Platform	Knockout (NHEJ)	Electroporation			Strategic partnership & Collaboration
Allogeneic-T Platform	Knockout (NHEJ)	Electroporation			Strategic partnership & Collaboration

제넥신기술의 성과 : 유한양행-베링거인겔하임 Deal

디지털타임스

유한양행, 베링거에 1조 규모 기술수출...비알콜성 지방간염 신약 후보물질(상보)

기사입력 2019.07.01. 오전 8:25 기사원문 스크랩 본문듣기 · 설정

공감 댓글

요약본 Beta 가

유한양행은 베링거인겔하임에 NASH(비알콜성 지방간염)을 치료하기 위한 융합단백질 (GLP-1/FGF21 dual agonist)을 기술수출하는 계약을 체결했다고 1일 공시했다. 총 기술수출 금액은 약 1조53억원(8억7000만달러) 규모다.

반환 의무 없는 계약금은 4000만달러로, 이중 1000만달러는 GLP-Tox(비임상 독성시험) 이후 수령할 예정이다.

마일스톤 기술료는 개발, 허가, 매출에 따른 단계별 마일스톤 총 83000만 달러를 수령하게 된다. 마일스톤 기술료는 조건 달성시 수령할 수 있다.

또한 경상기술료(Royalty)는 순매출액에 따라 수령할 예정이다.

유한양행은 총 기술수출금액의 5%를 제넥신에 지급할 예정이다. 'YH25724'로 불리는 이 후보물질은 유한양행이 2015년 국내 바이오 벤처인 제넥신에서 도입한 '체내 지속형 바이오신약 기술'을 활용해 개발 중이다. 올해 연말쯤 미국 FDA(식품의약품)에 IND(임상시험계획)를 제출한다는 게 유한양행의 계획이다.

이번 계약의 대상 지역은 한국을 제외한 전 세계다.

재무계획 (Cash Inflow)

Genexine

ToolGen

2022년까지의 가용현금

약 5,000억원

300억원

1,800억

1,350억

1,755억

+

300억

보유
현금성자산

투자지분 가치
실현 가능액

기술이전
수입 가능액

보유
현금성자산

- 충분한 가용현금 확보가능
- 차세대 글로벌 경쟁력 확보를 위한 Strategic Convergence가 필요



**Genexine
ToolGen
합병**

- 향후 3년간 통합법인의 개발 소요금액 충족
- 미래 신기술 확보를 위한 적극적 투자 및 연구개발비 집행 가능
- FI를 통한 추가 자금조달은 당분간 불필요 (기존 주주의 Dilution를 최소화)
- 전략적 투자자(SI)와의 전략적 차원의 자본참여는 열려있음

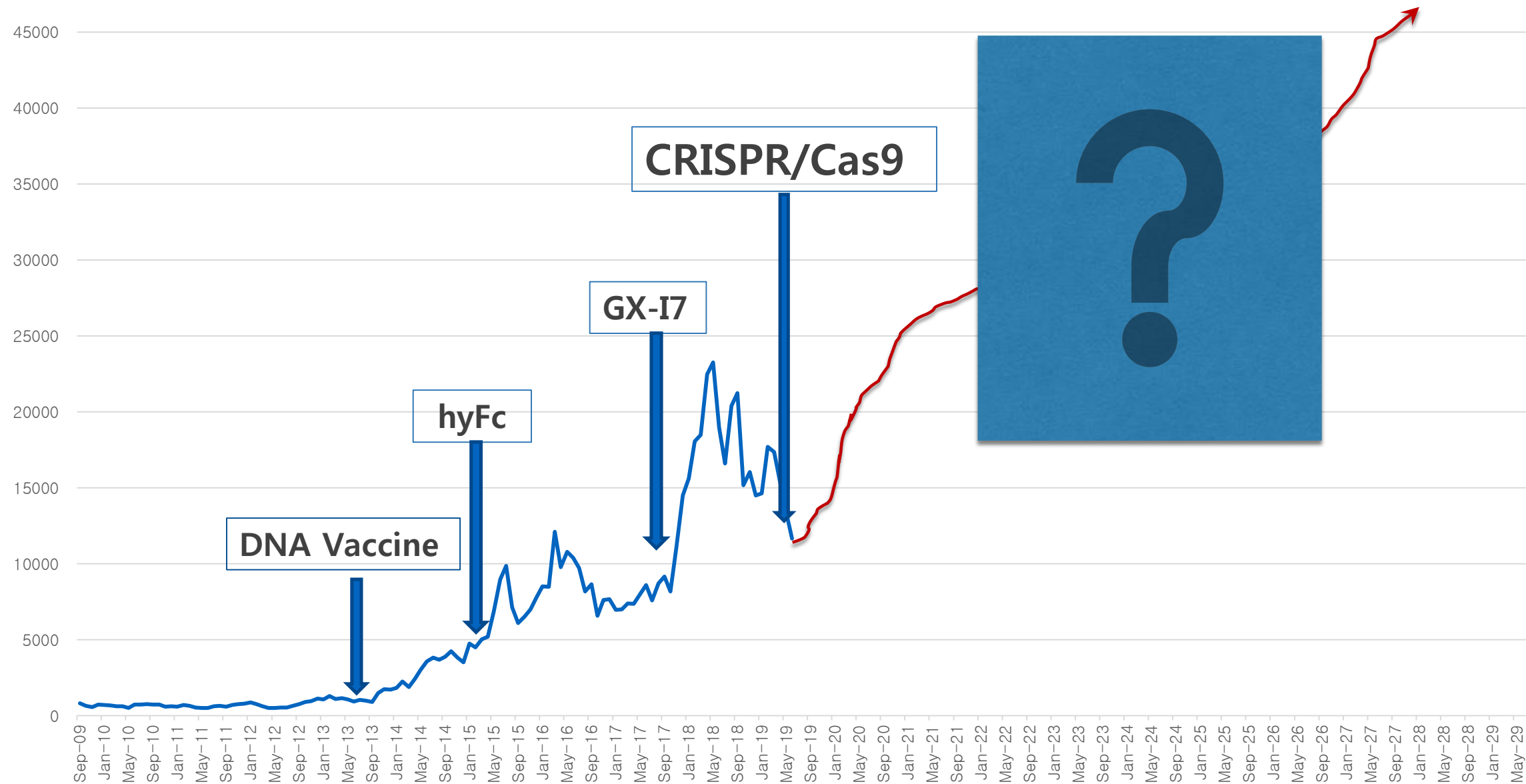
재무계획 (Cash Outflow)

(단위 : 억원)

2020~2022 분야별 기금 소요 계획	
Cash Outflow	미래신기술 창출 투자 4,000
	연구개발비 1,535
	- 신기술 (CAR-T, Cell/Gene Therapy) 620
	- 혁신신약 (Early Stage) 300
	- 혁신신약 (Preclinical) 150
	- 개량신약 (Late/Early Stage) 150
	- 연구 인건비 315
	인건비 135
	관리비 610
	연구 인프라 구축 590
Total Cash Outflow (-) 7,870	

- R&D 전략위원회 4,000억 장기적 성장을 위한 투자**
 - 내부 창출 현금 1,000억
 - 전략적 투자자(SI)와의 전략적제휴를 통한 3,000억 가용
 - 차세대 기술개발
 - M&A, 전략적 지분투자
 - Global License-Out의 기능 수행
- 연구개발비 1,535억**
 - 기존 개발 Pipeline 이외 우선순위
 - ❖ Bi-Specific AB
 - ❖ In Vivo Gene Therapy
 - ❖ Ex-Vivo (CAR-T, DC Vaccine)
- 인건비(R&D이외)/관리비 745억**
 - CRISPR/Cas9 특허보호 비용 350억 포함 (향후 특허 Interference, Opposition에 따라 증감 예상)

ToolGenexine Market Cap



End of Presentation

Thank You!