



SUCCESS FACTORS FOR GLOBAL BIOCLUSTERS

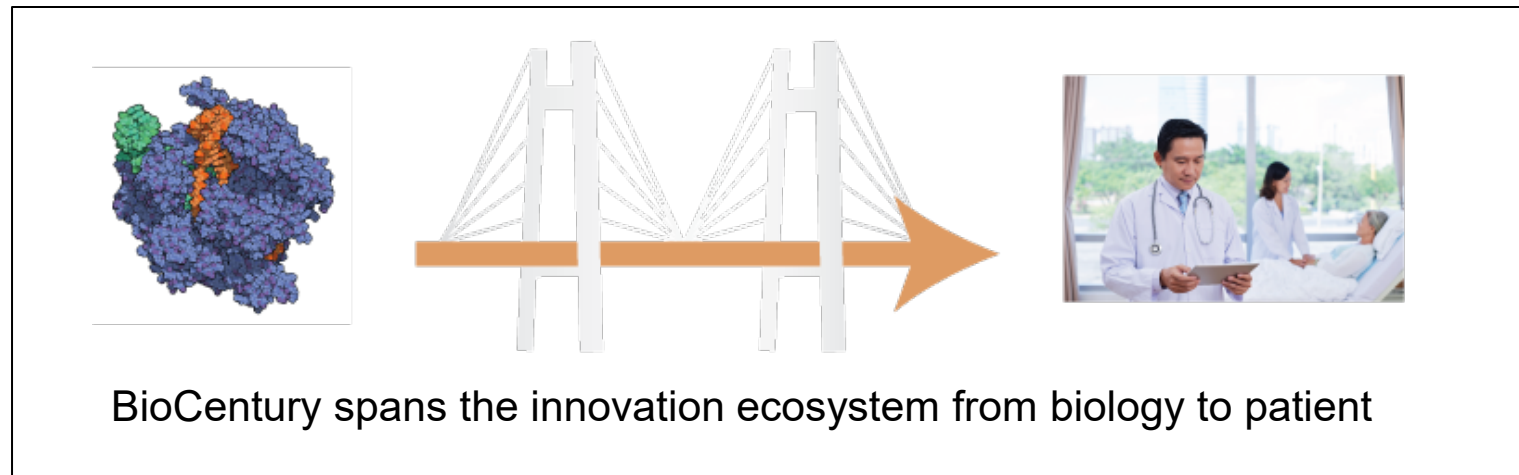
Global Bio Forum

Seoul, November 26, 2019



BioCentury: A Business Intelligence Platform

BioCentury provides a continuous stream of business and scientific analysis, comprehensive biopharma industry data, executive conferences and research services for biopharma companies, investors, academia and government.



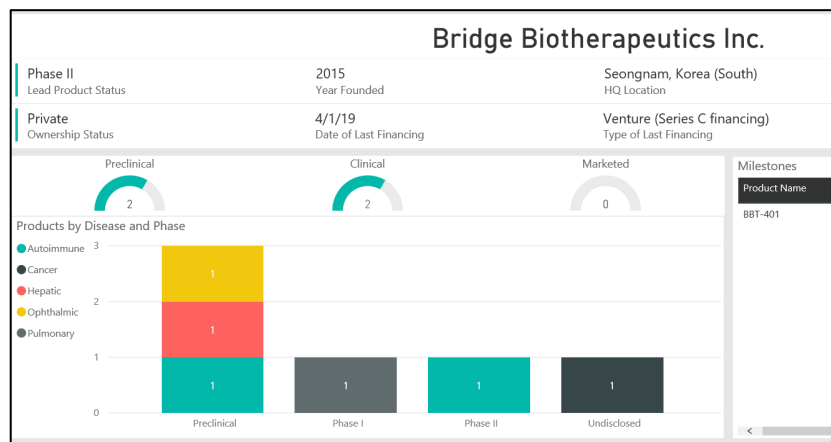
BioCentury's Use Case

BioCentury's independent analysis, data, conferences and people can be employed to help answer critical questions, decipher strategic trends, objectively evaluate product and business strategies, and evaluate financial and policy risk faced by science-based companies.



BioCentury Combines Data AND Context

BCIQ's Company, Product and Target Profiles are directly linked to the relevant BioCentury analysis



BioCentury July 18, 2019

BIOCENTURY

PREVIOUS ARTICLE | BACK TO FEED | NEXT ARTICLE

SHARE | TEXT SIZE | PRINT | SAVE ARTICLE

Boehringer deal enables Bridge to expand pipeline, take in-house programs further

BY SANDI WONG, STAFF WRITER | JUL 18, 2019 | 1:58 PM PDT

EDITOR'S NOTE: THIS ARTICLE WAS UPDATED ON JUL 18, 2019 AT 3:53 PM PDT

In the latest sign of success for Bridge's strategy to translate first-in-class compounds discovered in Asia, the Korean biotech granted **Boehringer** exclusive global rights to its lung fibrosis candidate **BBT-877**.

The partnership is the second out-licensing deal for Bridge Biotherapeutics Inc. (Seongnam, South Korea), which in-licenses late discovery molecules primarily from academic institutions, biotechs or pharmas in South Korea, China and Japan; develops them through Phase IIa proof-of-concept; and then partners the compounds (see "**Pellino Play**").

Boehringer will pay Bridge €45 million (\$50.6 million) up front. The biotech is eligible for €1.1 billion (\$1.2 billion) in milestones, plus royalties up to double digits. The partners will collaborate to develop **BBT-877**, an inhibitor of ENPP2, to treat fibrosing interstitial lung diseases; the first indication will be idiopathic pulmonary fibrosis (IPF). **BBB-877** is in Phase I testing; a Phase II trial is due to begin within 12 months.

Guided by a Top Scientific Advisory Board

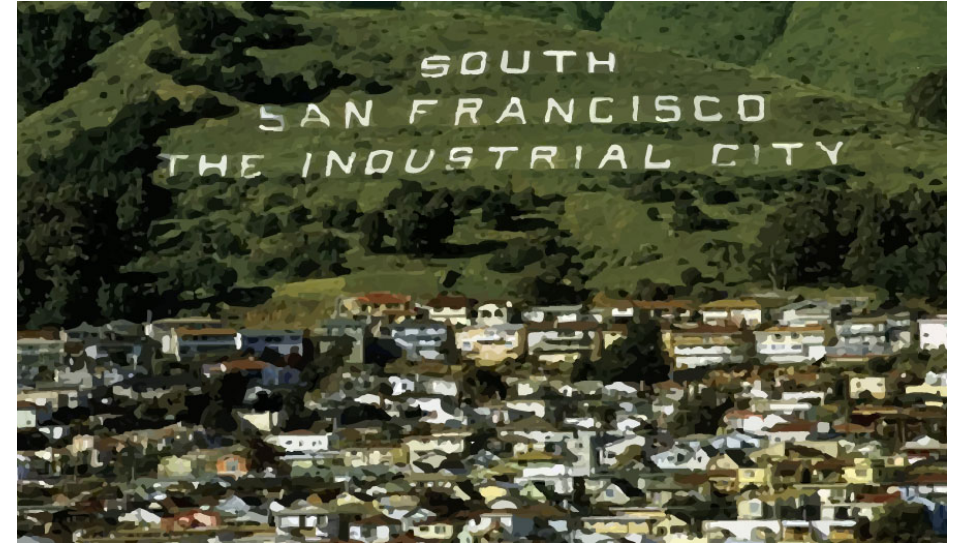
- ❑ **Mark Currie, Ph.D.**, President and CSO, Cycleron Therapeutics Inc.
- ❑ **Francis Cuss, M.D.**, (former) EVP and CSO, R&D, Bristol-Myers Squibb Co.
- ❑ **Susan Dillon, Ph.D.**, Co-Founder, President and CEO of Aro Biotherapeutics Co.
- ❑ **Laurie Glimcher, M.D.**, President and CEO, Dana-Farber Cancer Institute
- ❑ **Michael Hayden, M.D., Ph.D.**, (former) President Global R&D, CSO, Teva Pharmaceutical Industries Ltd.
- ❑ **Iya Khalil, Ph.D.**, Chief Commercial Officer and Co-Founder, GNS Healthcare Inc.
- ❑ **Menelas Pangalos, Ph.D.**, EVP, BioPharmaceuticals R&D, AstraZeneca plc
- ❑ **Andrew Plump, M.D., Ph.D.**, President of R&D, Takeda Pharmaceutical Co. Ltd.
- ❑ **James Sabry, M.D., Ph.D.**, Global Head of Pharma Partnering, Roche
- ❑ **Paul-Peter Tak, M.D., Ph.D.**, CEO, Kintai Therapeutics Inc.; Venture Partner, Flagship Pioneering
- ❑ **Douglas Williams, Ph.D.**, Co-founder, President and CEO, Codiak BioSciences Inc.
- ❑ **Jan van de Winkel, Ph.D.**, President and CEO, Genmab A/S
- ❑ **Elias Zerhouni, M.D.**, (former) President, Global R&D, Sanofi

SAB Venture Members

- ❑ **Houman Ashrafian, D.Phil.**, Managing Partner, SV Health Investors
- ❑ **Bruce Booth, D.Phil.**, Partner, Atlas Venture
- ❑ **Francesco de Rubertis, Ph.D.**, Co-founder and Partner, Medicxi
- ❑ **Todd Foley**, Managing Director, MPM Capital
- ❑ **Carol Gallagher, Pharm.D.**, Partner, New Enterprise Associates
- ❑ **Reid Huber, Ph.D.**, Partner, Third Rock Ventures
- ❑ **Kewen Jin, M.D.**, Managing Partner, Serica Partners
- ❑ **Sophie Kornowski, Ph.D.**, Senior Partner, Gurnet Point Capital
- ❑ **Antoine Papiernik**, Managing Partner, Sofinnova Partners
- ❑ **Kush Parmar, M.D., Ph.D.**, Managing Partner, 5AM Ventures
- ❑ **Cary Pfeffer, M.D.**, Partner, Third Rock Ventures
- ❑ **Otello Stampacchia, Ph.D.**, Founder and Managing Director, Omega Funds

Today's Discussion

- ❑ Picturing today's hubs
- ❑ Inflection points in the Bay Area and Boston
- ❑ Essential characteristics of innovation hubs
- ❑ Revisiting some thoughts about Korea



Source: BioCentury & Getty Images

Take-Home Messages

- ❑ There is no formula



- ❑ Checking the boxes isn't sufficient



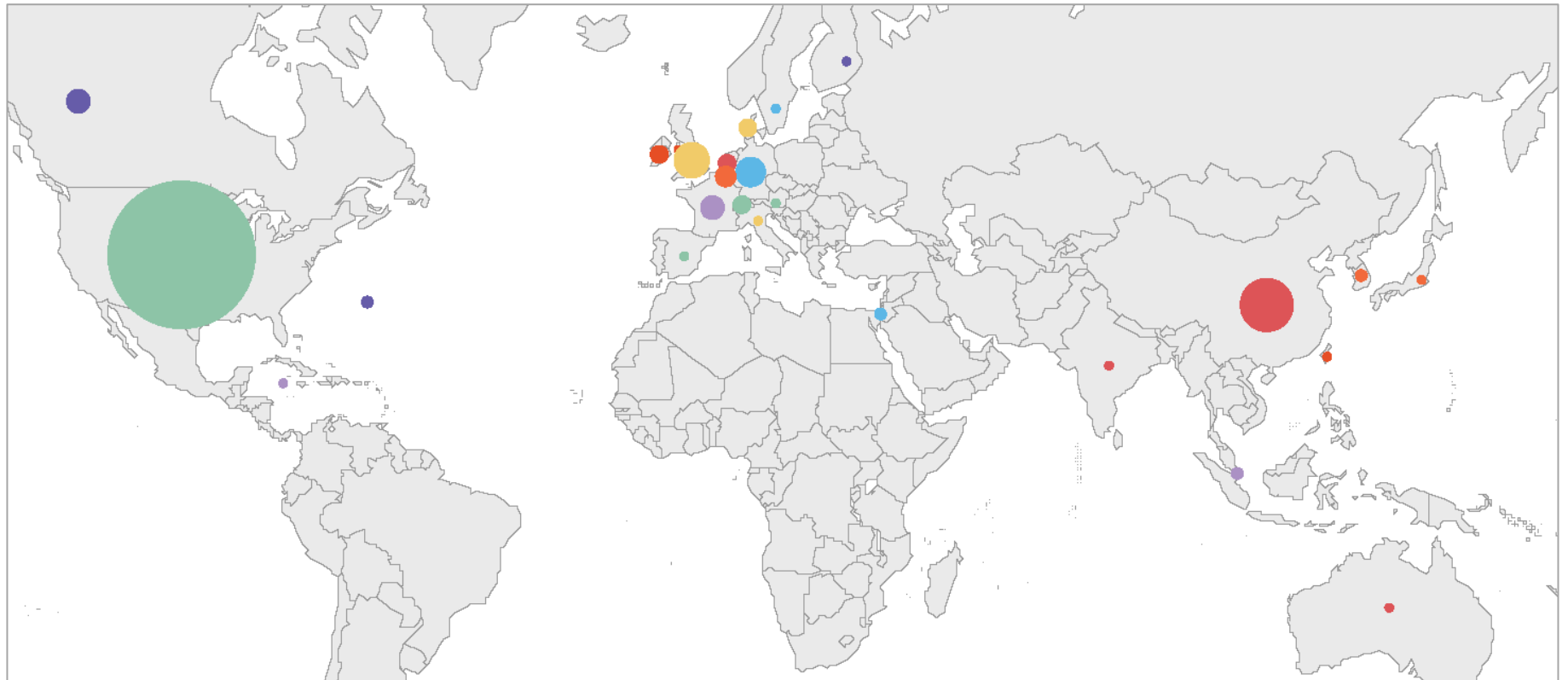
- BUT innovation centers do share essential characteristics

The Desired Outcomes

- ❑ Magnets for smart money
- ❑ Destinations for the best people

Amount of seed and Series A financing since beginning of 2016.
BioCentury, Oct. 17, 2019

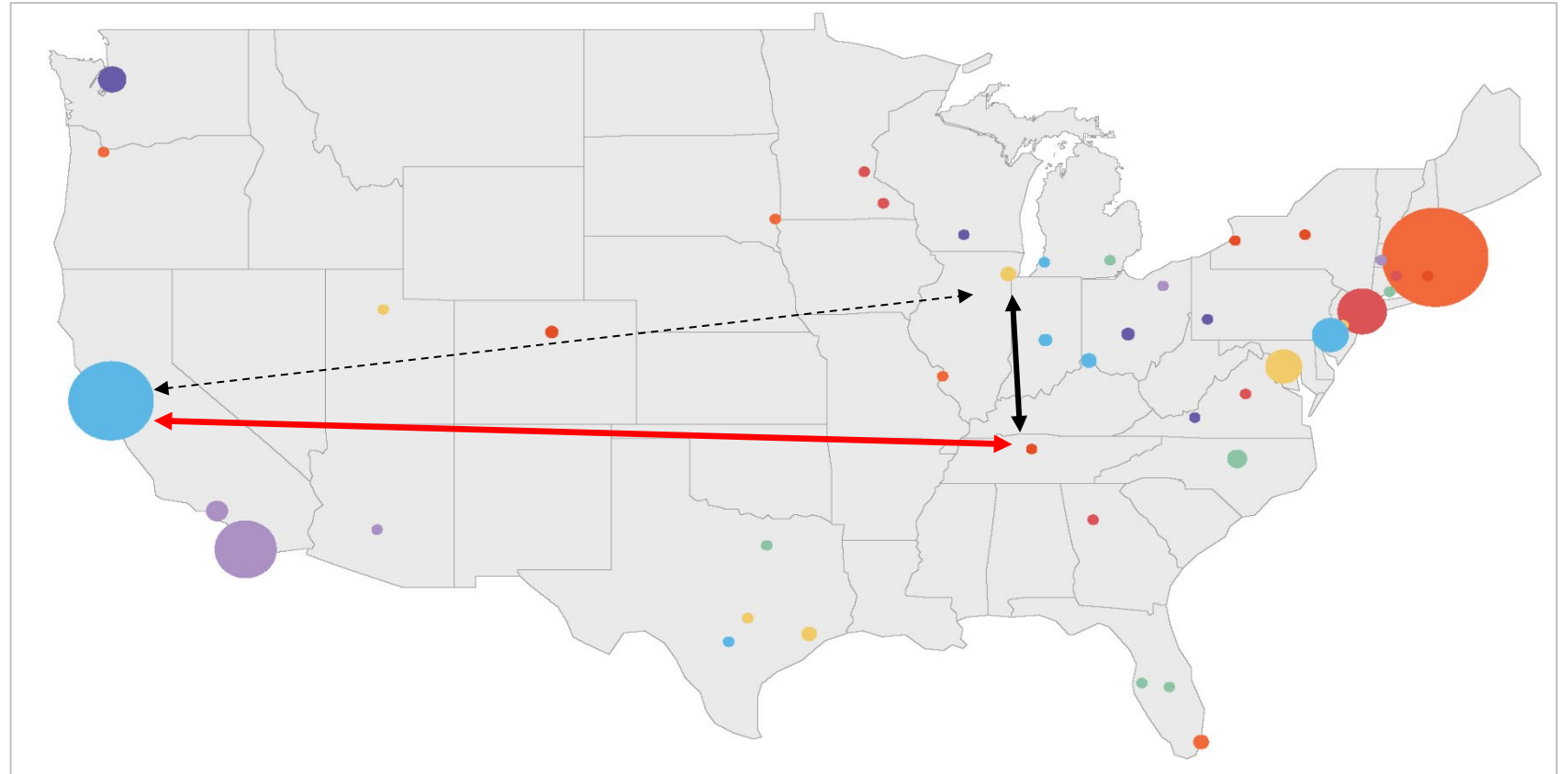
- U.S. \$13.3B
- China \$1.7B
- UK \$722M



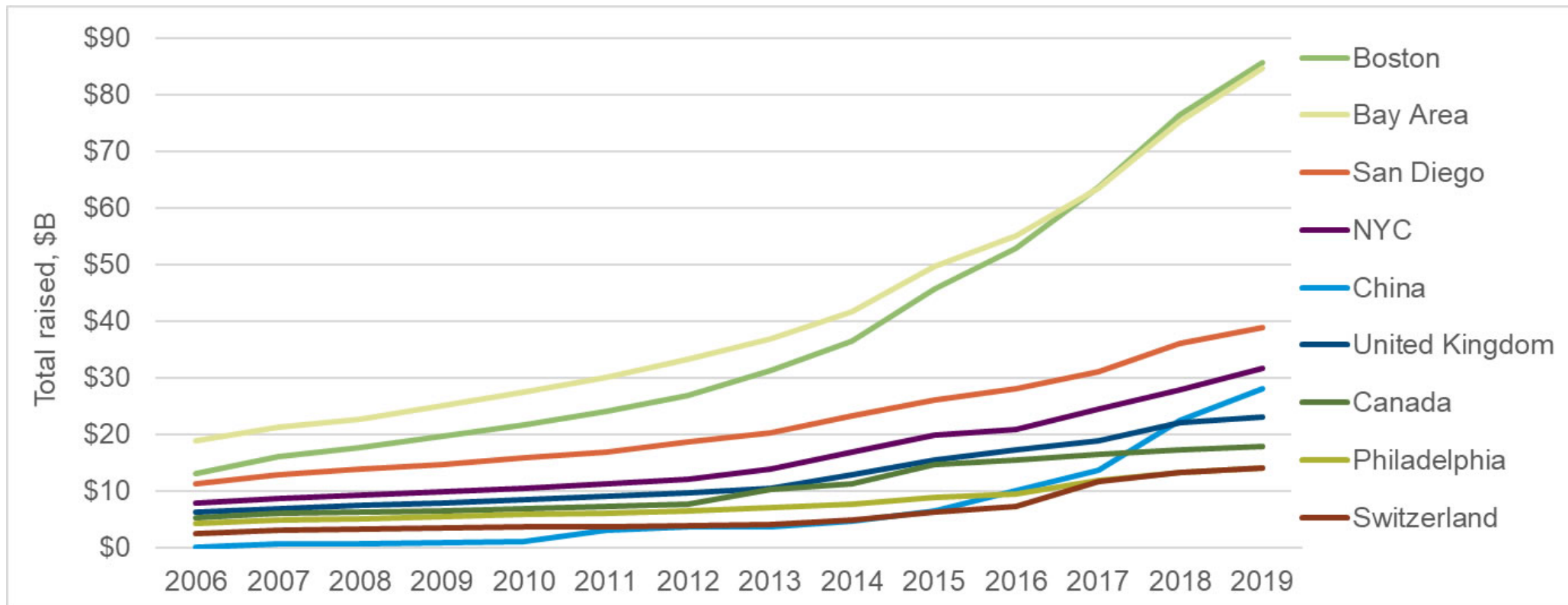
Seeing Both Sides

- ❑ Boston \$4.8B
- ❑ Bay Area \$3.1B
- ❑ San Diego \$1.5B
- ❑ New York \$1.0B
- ❑ Chicago \$89M (#11)
- ❑ Nashville \$13M (#34)

Seed and Series A financing
since the start of 2016.
BioCentury, Oct. 17, 2019



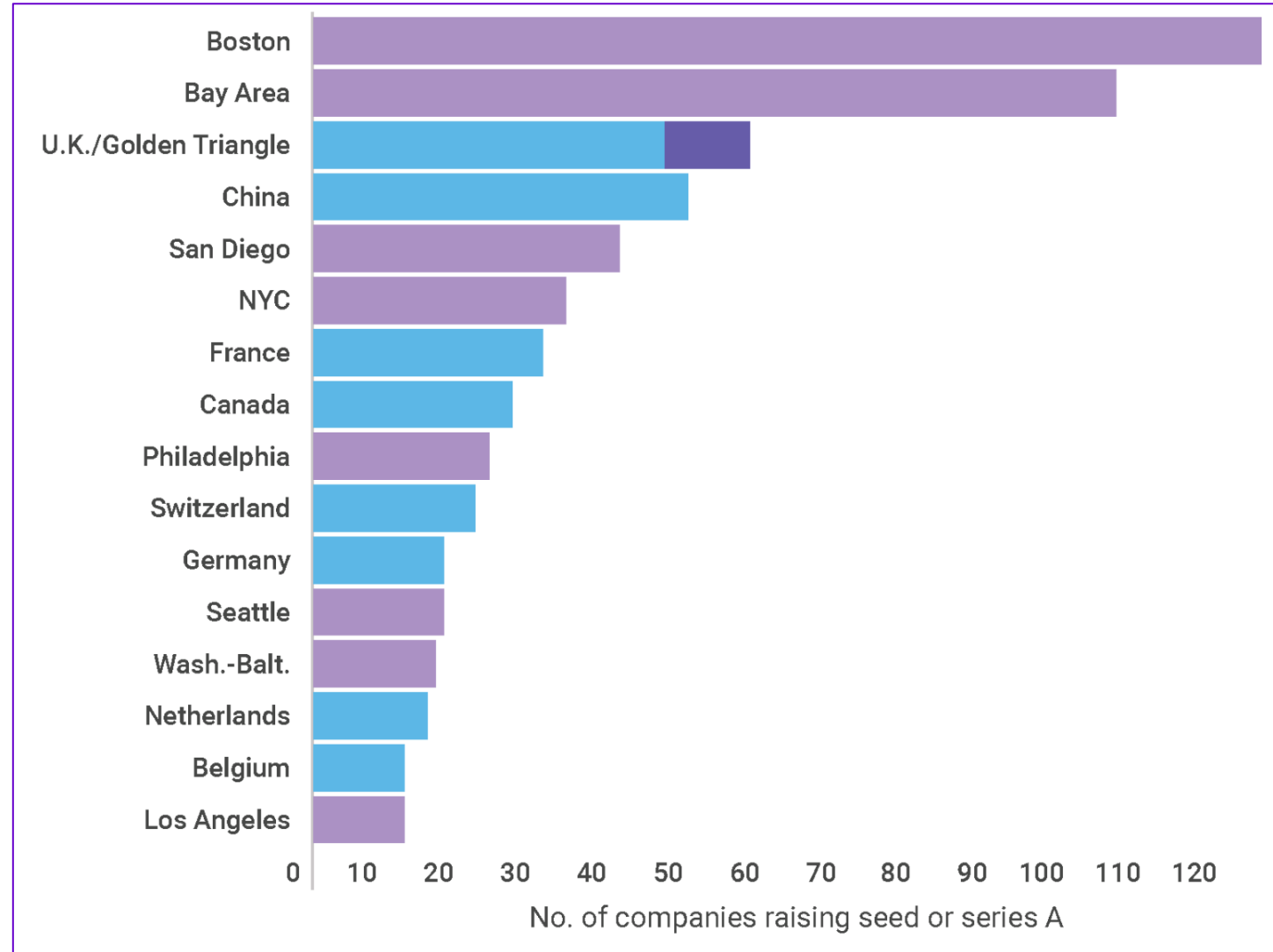
Red-Shift: The Biggest Centers Race Away



Cumulative biotech equity raised since 2016. Includes therapeutics and diagnostics, drug delivery devices, digital therapeutics, and selected biopharma service companies; excludes debt and convertible stock. Source: BCIQ

Start-Up Centers

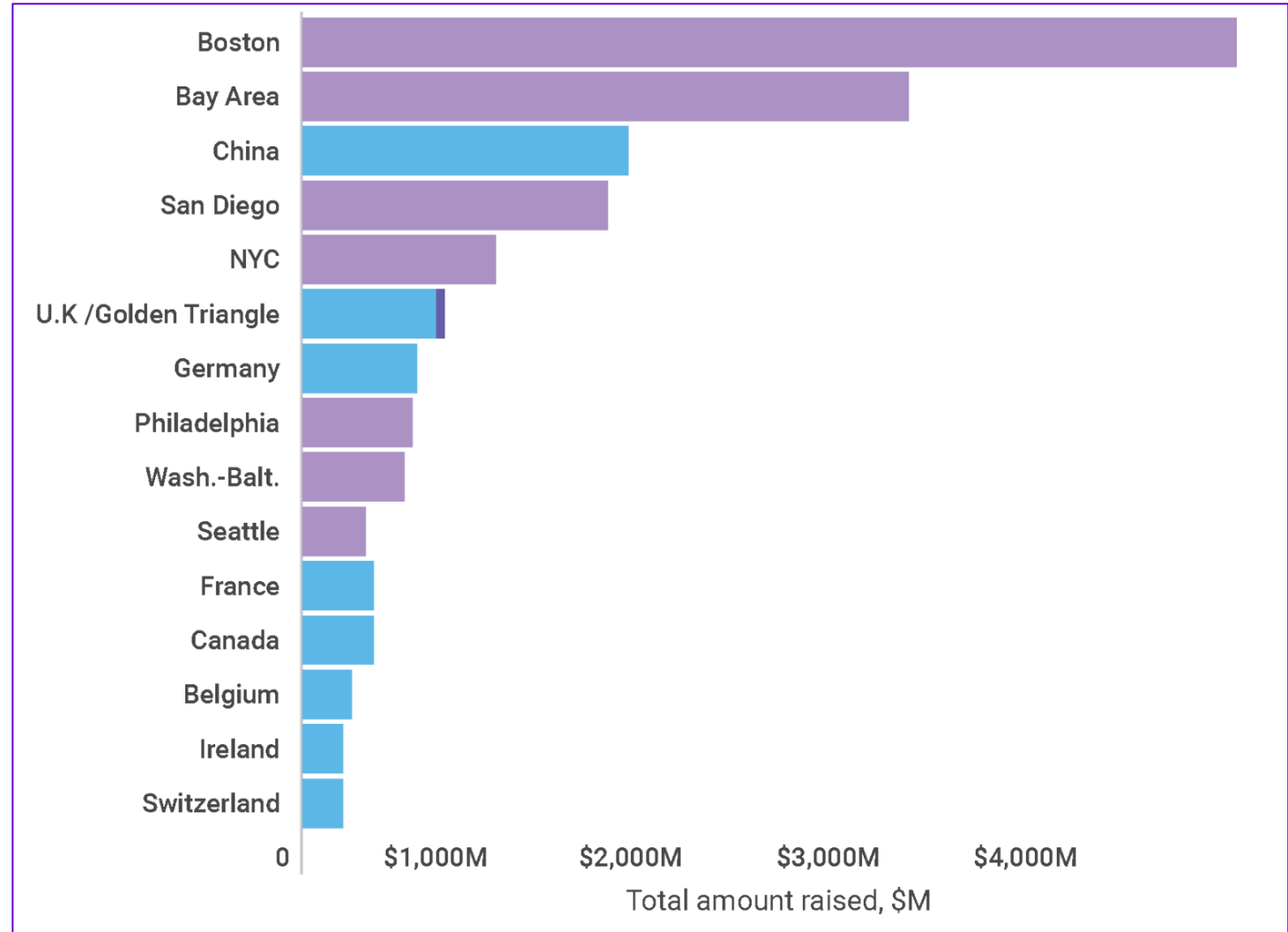
of companies raising seed and series A financing since the start of 2016



BioCentury, Oct. 17, 2019

Early Stage Money

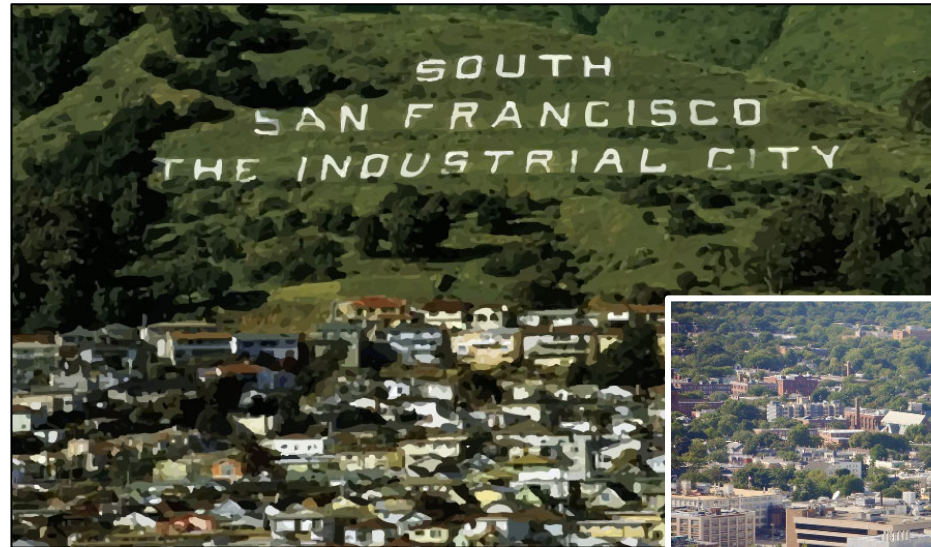
Money raised in seed and Series A financing since the start of 2016



BioCentury, Oct. 17, 2019

Foundational History

- ❑ Infrastructure
- ❑ Technology
- ❑ Companies
- ❑ People
- ❑ Events



Source: BioCentury & Getty Images



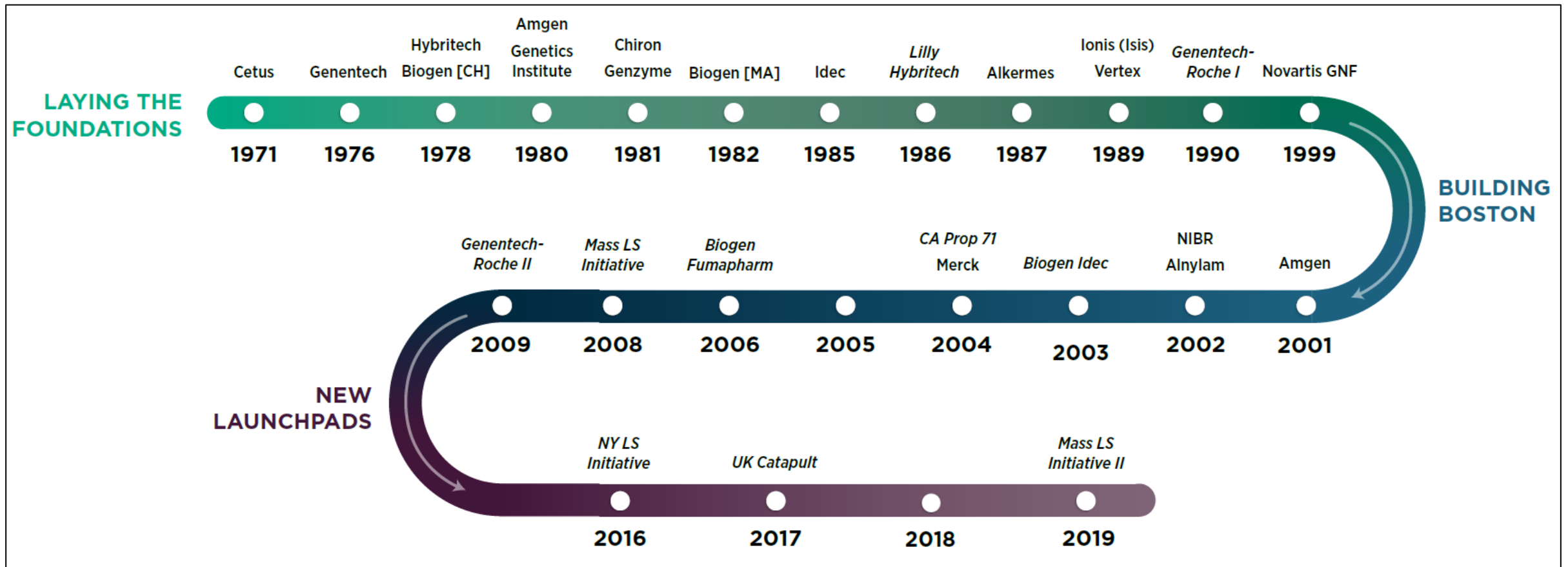
What Matters Most?

- Top academic institutions?
- Government support?
- Breakthrough technology?
- Access to capital?
- Access to talent?
- Convenience of access?
- Room to build?



Source: BioCentury & Getty Images

‘Double, Double, Toil and Trouble’



Preconditions for Thriving Innovation Ecosystems

- ❑ Enabling professional infrastructure
- ❑ Expert, experienced capital
- ❑ Long-term leadership



Henri Termeer with Genzyme employees.
BioCentury, May 19, 2017. Source: Sanofi Genzyme

Behaviors in Thriving Innovation Ecosystems

- ❑ Driven by personal accomplishment
- ❑ Superhuman stamina
- ❑ Manages risk, not failure



Phenotype of Thriving Innovation Ecosystems

- ❑ Density
- ❑ Career mobility
- ❑ Robust information exchange
- ❑ Multidisciplinary
- ❑ Translational excellence
- ❑ Fecund



Source: NIH

Building Brand in the Ecosystem

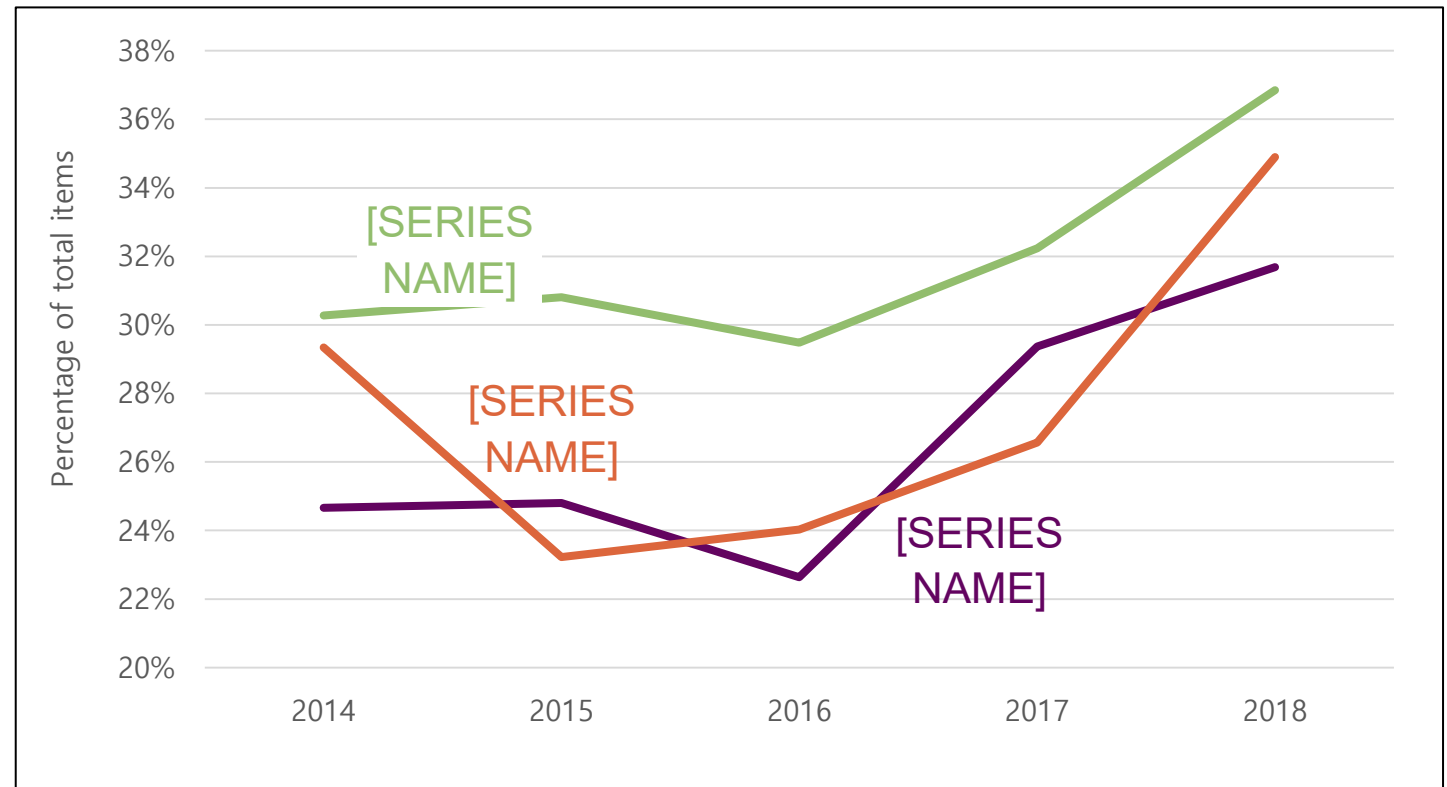
- ❑ Deliver successes
- ❑ Be internationally relevant
- ❑ Be visible in the global innovation network



Global Relevance: Hot Topics Activity

% of translational findings in each region

- Gene therapy
- Cell therapy
- Gene editing
- Nucleic acid technologies
- Microbiome
- Oncolytic viruses
- Neuroinflammation
- AMR

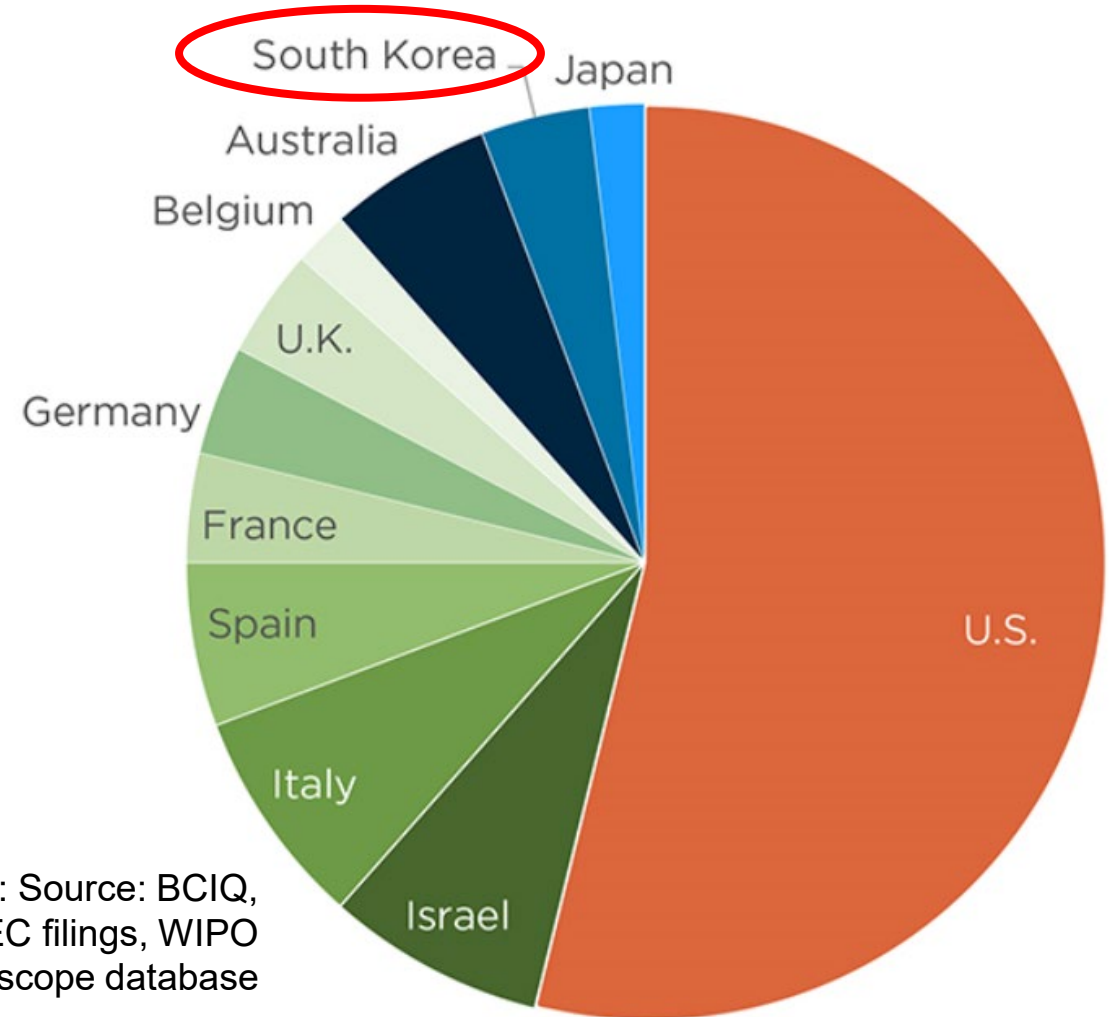


Europe's best bet for closing the gap with the U.S. is to nurture its hotspots of innovation for new modalities. *BioCentury*, May 24, 2019

New Modality Origins

Sources of 52 products in new modalities-- gene therapies, cell therapies, and antisense oligonucleotides

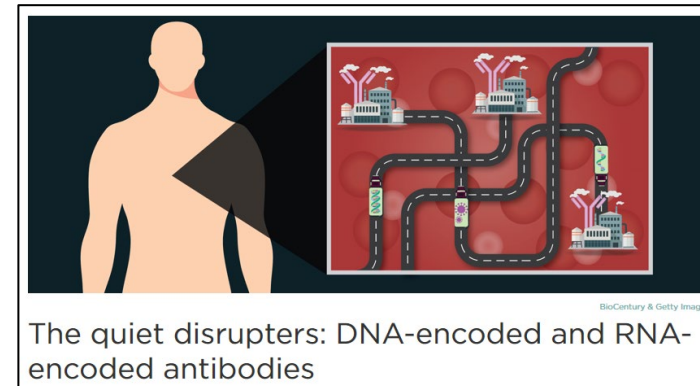
- ❑ Approved since 2015 or in Phase III or later
- ❑ U.S. 53%
- ❑ Europe 35%
- ❑ Asia-Pacific 12%



BioCentury, May 24, 2019: Source: BCIQ, company websites, SEC filings, WIPO Patentscope database

Needed Now: ‘Outside the Box’ Thinking in Manufacturing

Company	Product/platform	Target	Modality	Status	Indication
RegenxBio	RGX-314	VEGF	AAV8	Ph I/II	Wet age-related macular degeneration (AMD)
Adverum	ADVM-022	VEGF	AAV7	Ph I	Wet AMD
Moderna	MRNA-1944	Unknown antigen of chikungunya virus	RNA	Ph I	Chikungunya
BioNTech	BNT141	Undisclosed	RNA	Preclin	Solid tumors
BioNTech	BNT142	CD3 and Claudin 6 (CLDN6)	RNA	Preclin	Solid tumors
CureVac	RNA-encoded antibodies	Multiple	RNA	Preclin	Rare diseases
ethris / AstraZeneca	Stabilized Non-Immunogenic mRNA (SNIM RNA)	Multiple	RNA	Preclin	Asthma, COPD, idiopathic pulmonary fibrosis
Inovio	DNA-encoded monoclonal antibody technology (dMAb)	Multiple	DNA	Preclin	Chikungunya, dengue, HIV, influenza, Zika
Voyager / AbbVie	Vectorized antibody	α synuclein or tau	AAV or other virus capsid	Preclin	Alzheimer's disease (AD), Parkinson's disease, other synucleinopathies and tauopathies



How delivering antibodies via DNA or RNA could disrupt the biologics business. BioCentury, Nov. 21, 2019

What's Next: Digital Rx, Dx & Disease Management Tools



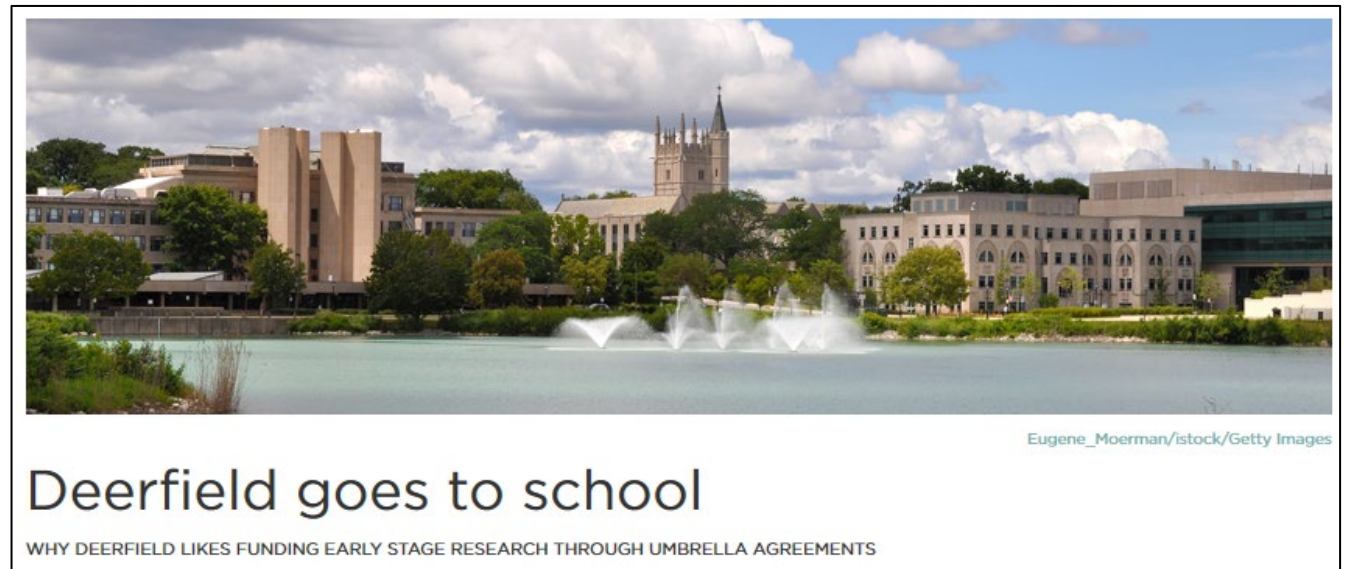
Rules of the road for digital health.
BioCentury, Aug. 30, 2019

COMPANY	DIGITAL INITIATIVES				
	IN-HOUSE PROGRAMS	CLINICAL TRIALS	DEALS	INVESTMENTS	CHIEF DIGITAL OFFICER
AbbVie	✓	✓	✓		
Amgen	✓		✓	✓	✓
Astellas	✓		✓	✓	
AstraZeneca	✓	✓	✓	✓	
Bayer	✓	✓	✓	✓	
Biogen	✓	✓	✓		
Boehringer Ingelheim	✓	✓	✓	✓	✓
Bristol-Myers Squibb		✓	✓		
Celgene					✓
Eisai	✓		✓	✓	✓
Eli Lilly	✓	✓	✓		✓
Gilead Sciences		✓	✓		
GlaxoSmithKline	✓	✓	✓		✓
Johnson & Johnson		✓	✓	✓	
Merck & Co.	✓	✓	✓	✓	
Merck KGaA	✓	✓	✓	✓	✓
Novartis	✓	✓	✓	✓	✓
Novo Nordisk	✓	✓	✓		
Otsuka	✓	✓	✓	✓	
Pfizer		✓	✓	✓	✓
Roche/Genentech	✓	✓	✓	✓	
Sanofi	✓	✓	✓	✓	✓
Shionogi		✓	✓		
Takeda	✓	✓	✓		✓
UCB		✓	✓	✓	

Innovation is Happening Everywhere

Deerfield aiming to fund 50 molecules in 3-4 years via growing academic network

- ❑ NYC incubator \$635M
- ❑ Columbia \$130M
- ❑ Harvard \$100M
- ❑ Dana Farber \$80M
- ❑ Johns Hopkins \$65M
- ❑ Northwestern \$65M
- ❑ UCSD \$65M
- ❑ Vanderbilt \$65M
- ❑ Broad Institute \$50M



Why Deerfield likes funding early stage research through umbrella agreements, BioCentury, June 7, 2018. Source: Getty Images

The Importance of Innovation Networks

- ❑ Translation requires networks of academics + strategic investors + venture investors
- ❑ Networks create value
 - Trust
 - Credibility
 - Relevance
 - Visibility
 - Access to ideas
 - Access to skills
 - Access to deals



Why Third Rock likes multi-asset option deals for its newcos.
 BioCentury, Dec. 20, 2018. Source: BioCentury & Getty Images

Membership in Networks Will Build Korea's Brand



Communicate Through BioCentury

BioCentury is a platform for extending Korea's reach into the international innovation ecosystem

- ❑ Providing visibility and validation for Korean innovators
- ❑ Backed by data and analytical content to inform Korean strategists

Questions?

David Flores, President & CEO: dflores@biocentury.com

Josh Berlin, Executive Director of Business Development: jberlin@biocentury.com

Matt Krebs, APAC Strategic Account Manager: mkrebs@biocentury.com

Collaborating to Share Knowledge in Korea



카드몬제약은 지난 11일(현지시간) 만성편대속주병(GvHD) 치료를 위한 후보물질 KD025를 대상으로 진행한 임상 2상 시험에 대한 중간분석 결과를 공개했다. © 뉴스1



미국 뉴욕에 본사를 둔 카드몬 제약(Kadmon Holdings Inc.)이 만성 이식편대속주병(GvHD) 치료를 위한 임상 2상 시험에서 1차 효능을 입증했다. 전체 데이터 분석이 완료되는 내년에는 품목허가 신청도 가능할 것으로 보인다.

카드몬 제약은 지난 11일(현지시간) 만성 GvHD로 인해 최소 2회 이상 전신요법을 받은 환자들을 위한 후보물질 KD025을 대상으로 진행중인 임상 2상 시험 중간분석에서 1차 주요 효능평가지표를 충족시켰다고 발표했다. 카드몬은 이번 임상 시험 결과가 나오는 내년쯤 신약 품목허가 신청서(NDA)를 제출할 계획이다.

BIOCENTURY

PREVIOUS ARTICLE	BACK TO FEED	NEXT ARTICLE
SHARE	TEXT SIZE	PRINT
Twitter LinkedIn Email	A A A	Print

Early success in GvHD sets up NDA for Kadmon's lead

BY SANDI WONG, STAFF WRITER | NOV 11, 2019 | 5:02 PM PST

With its lead clinical candidate meeting the primary endpoint at an interim analysis of a pivotal trial for third-line chronic graft-versus-host disease, Kadmon plans to submit an NDA next year for KDO25 following next half's primary analysis.

On a conference call Monday, Kadmon Holdings Inc. (NYSE:KDMN) President and CEO Harlan Waksal said the p-associated coiled-coil containing protein kinase 2 (ROCK2) inhibitor cleared a hurdle "much higher than the primary analysis" by meeting at this stage.

While the primary endpoint evaluates overall response rate (ORR) at six months after the last patient was enrolled, Waksal said the company had previously agreed with FDA that KDO25's efficacy would be determined by "best response at any time." Because the interim analysis covers all the trial's patients, the best ORR will not decrease.

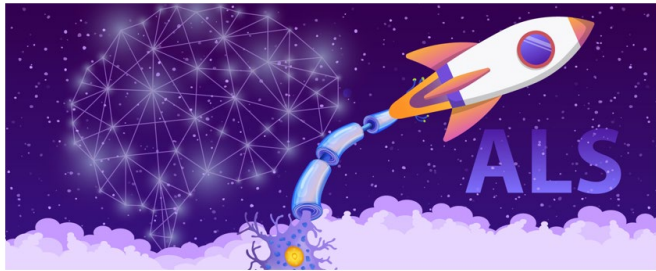
Kadmon, which reported the data late Monday, was up \$0.61 (20%) to \$3.60 in after-hours trade.

BioCentury Facts

- ❑ BioCentury analysts create ORIGINAL content using primary resources
- ❑ BioCentury's independent analysis and data provide a framework for business decisions by science-based companies, investors and institutions
- ❑ Informed by a strong Scientific Advisory Board
- ❑ Founded 1992 with backing of angels in venture capital, biotech and investment banking
- ❑ Headquarters: Redwood City, California
- ❑ Employees in U.S., U.K., the Netherlands
- ❑ Privately held
- ❑ Turnover: North America 55%, Europe 30%, Asia 15%

BioCentury Synthesizes Pipeline Progress

BioCentury Analysts use BCIQ and original research to visualize and analyze disease pipelines



ALS platform trial to put neuroinflammation hypothesis to the test

Company	Product	Description	Target	Mechanism
Biohaven Pharmaceutical Holding Co. Ltd. (NYSE:BHVN)	Verdiperstat (BHV3241)	Small molecule myeloperoxidase (MPO) inhibitor	MPO	Neuroinflammation
Clene Nanomedicine Inc.	CNM-Au8	Orally administered nanocrystalline gold suspension	NA	Neuroprotection
Implicit Biosciences Ltd.	IC14	mAb against CD14	CD14	Neuroinflammation
Prilenia Therapeutics	Pridopidine	Sigma-1 receptor agonist	Sigma-1 receptor	Neuroprotection
Ra Pharmaceuticals Inc. (NASDAQ:RARX)	Zilucoplan	Macrocyclic peptide inhibitor of complement C5	C5	Neuroinflammation

The idea that neuroinflammation drives ALS will get its most rigorous test to date with the Healey ALS platform trial.. BioCentury, Oct. 3, 2019

BioCentury Deciphers Therapeutic Trends

BioCentury identifies key drivers of success in emerging therapeutic spaces

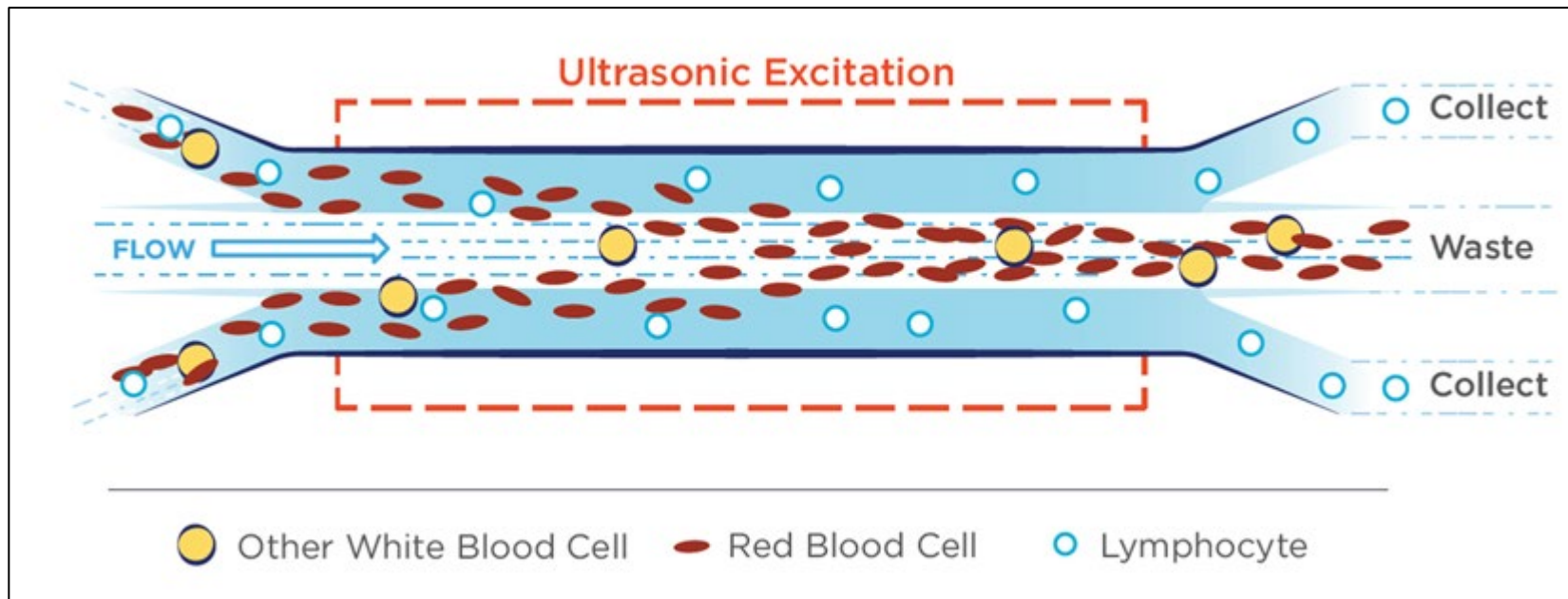


Vertex's triple therapy will reshape the landscape of cystic fibrosis trials

Trikafta approval extends Vertex's dominance, pushes competitors to rare mutations and new trial designs, BioCentury Nov. 1, 2019

Company	Compound	Ph	Population	ppFEV1	Time	N	p-Value
Vertex	Trikafta	III	Het ΔF508	13.8%	Week 4	403	p<0.0001
				14.3%	Week 24	403	p<0.0001
		III	Hom ΔF508 (vs Symdeko)	10.0%	Week 4	107	p<0.0001
	Orkambi	III	Hom ΔF508	3.0%	Week 24	559	p<0.0001
		III	Hom ΔF508	2.6%	Week 24	549	p=0.0003
	Symdeko	III	Hom ΔF508	4.0%	Week 24	504	p<0.0001
			Het ΔF508 res mutation	6.8%	Week 4&8 average	244	p<0.0001
Translate Bio	MRT5005 8mg	I/II	Class I or II mutations	3.6%	Day 3	3	N/A
	MRT5005 16mg			11.6%	Day 3	3	N/A
	MRT5005 24mg			6.8%	Day 3	3	N/A
Galapagos	GLPG2737 + Orkambi	II	Hom ΔF508	3.4%	Week 4	22	p=0.08
Proteostasis	PTI-801 + PTI-808	I/II	Hom ΔF508	8.3%	Day 7	5	p=0.026
	PTI-801 + PTI-808 + PTI-428	I/II	Hom ΔF508	5.0%	Week 2	27	p<0.001

BioCentury Identifies Drivers of Emerging Technologies



How the non-profit Draper Laboratory is betting microfluidic and acoustic devices will lower barriers to cell therapy manufacturing. BioCentury, Oct. 31, 2019. Source: The Charles Stark Draper Laboratory

BioCentury Identifies Emerging Companies

Every week, BioCentury adds to its library of 2,000 profiles of emerging biotechs around the world

The illustration, set against a dark purple background, depicts various business and financial concepts. On the left, a woman in a yellow top sits on a large yellow lightbulb, holding a document. Next to her is a woman in a white lab coat sitting on a laptop. In the center, a man in a purple shirt stands on a ladder pointing at a line graph on a screen. To the right, a woman in a white shirt sits on a stack of gold coins. A white rocket ship is positioned in the background. The text 'EMERGING COMPANY PROFILE' is written in white at the top of the illustration. A small credit line 'BioCentury & Getty Images' is visible at the bottom right of the illustration.

Tmunity: Manufacturing momentum for CAR and TCR therapies

How Tmunity's focus on CMC for its engineered T cell therapies factored into its \$75M series B.
BioCentury, Oct. 31, 2019

BioCentury Monitors Regulatory Changes

BioCentury closely watches for regulatory transformations that could shape innovation

POLICIES THAT EITHER HINDER INNOVATION OR HURT PUBLIC HEALTH

CHINA	U.S.
Negative list for foreign investment into Chinese technologies	Strengthened FIRMMA/CFIUS reviews on minority foreign investments
Retightening of capital controls	
	Export control law may prevent exportation of certain foundational technologies
Tightening grants of 10-year business visas	Tightening grants of 10-year business visas
China Drug Administration Law and Patent Law fails to include IP reforms such as patent linkage and data exclusivity	
Restrictions on foreign access to genetic data	Restrictions on foreign access to genetic data
	NIH/FBI investigations into foreign funding of NIH scientists
Cybersecurity law that prevents encryption of data or communications inside China	

An overview of U.S. and China policies that could hurt both investment and innovation in biotech. BioCentury, Nov. 1, 2019

BIOCENTURY



THANK YOU!

