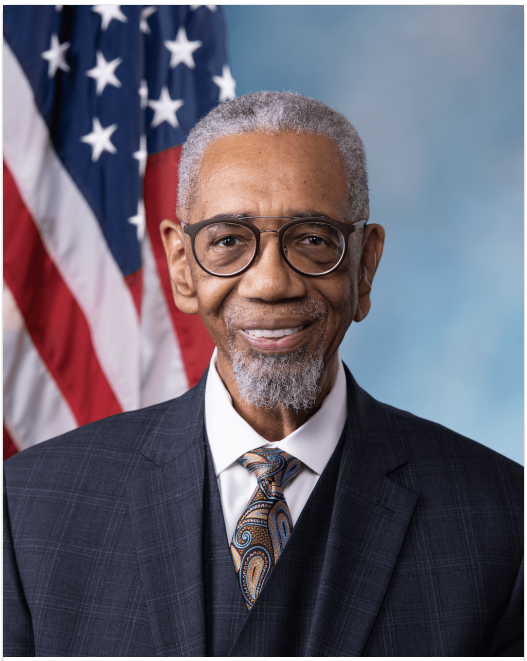
Zachary Brennan
Senior Editor

As thousands of clinical trials were suspended or halted due to the Covid-19 pandemic over the last year, House members on both sides of the aisle are trying to find new ways to jumpstart the clinical trial ecosystem.



Bobby Rush

For those with an IND in hand, and pursuing a drug targeting an unmet need, Reps. Bobby Rush (D-IL) and Brian Fitzpatrick (R-PA) on Friday introduced a new bill that would provide loans to continue such research if other funding sources run dry.

The representatives said the loans, known as BioBonds, would allow those with long-term, risk-averse capital such as pension funds and insurers, rather than those typically involved in such investments like VC or pharma companies, to invest up to $25 million per year in these trials.

Overall, the government would issue BioBonds worth up to $10 billion annually from FY 2022 through 2024, according to the bill's text.

"Millions of Americans suffering from cancer, Alzheimer's, and other terrible diseases cannot afford to wait for vital treatments and cures while clinical trials are disrupted," Rush said in a statement. "We must use every federal avenue to restart U.S. biomedical research and ensure that the clinical trials necessary to take basic research to the bedside receive the funding they urgently need."

Brian Fitzpatrick

The LOANS for Biomedical Research Act also includes a nod to greater diversity in clinical trials, noting that the HHS secretary should consider clinical trials that are "conducted by women researchers or researchers who are members of a racial and ethnic minority group or disabled."

More than a dozen non-profits, including Research! America, the Foundation Fighting Blindness, and the American College of Rheumatology, pledged their support for the bill.

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May 24, 2021 06:00 AM EDT

How Multi-Stakeholder Strategies for External Control Arms Can Overcome Approval and Access Barriers

Parexel Biotech

Leanne Larson, Senior Vice President & Head of Real-World Evidence and Access
Amy McKee, Head of Regulatory Oncology
Sangeeta Budhia, Global HTA Strategy Lead & Vice President, Pricing & Market Access
Parexel

The COVID-19 pandemic cast a spotlight on the expanding role of real-world evidence (RWE) and the window it provides into a more thorough understanding of the patient journey. Developers are finding

May 24, 2021 06:49 AM EDT
Updated 12:16 PM
People, In Focus

ENDPOINTS *in* FOCUS

Inside Vertex 3.0: Can Reshma Kewalramani repeat one of biotech's biggest success stories 'again and again and again'?

Jason Mast
Editor

This was not how Reshma Kewalramani imagined spending her first day as Vertex CEO. The 47-year-old nephrologist should've been in a spacious window office on the 14th floor of the biotech's glassy Boston Seaport headquarters, three rooms down from where she had spent the last three years. There should have been family photos on the desk, scientists buzzing in the labs beneath, and, feet away, executives she knew and trusted, briefing her on potential cures for sickle cell disease and diabetes.

Instead, on that bone-chillingly cold day last spring, she was at a makeshift desk in the dimly lit

business, medical and patient communities. "I scream it from the rooftops," says Bob Coughlin, former CEO of industry group MassBio, whose 19-year-old son has CF. "He's a whole new person, I'm filled with more gratitude than I've ever had in my whole damn life."

Now, just as Leiden passed the torch, the entire world was collapsing. It was a trial by wildfire for Kewalramani, who had already been an unlikely choice as CEO. The heads of large biotechs are almost exclusively businesspeople, executives whose chief job is to sell the drugs the company has already developed and find other companies to acquire. If they have MDs, they also have an MBA or 20 years of experience in sales. All, historically, have been men.

Kewalramani was a clear-eyed, affable physician who had trained at Boston's most prestigious hospitals and spent 12 years running trials at Amgen, but she had little experience on the business side of biotech. For the prior three years, leading Vertex's medical team, she stood opposite the executive committee at key moments, explaining results from trials she designed and ran in sickle cell and cystic fibrosis.

"She came from the medical side, which was unique," says Terry McGuire, founder and general partner of the Boston-based biotech VC Polaris Partners. "It speaks to their desire to really focus on what's going on in the clinic and for patients." Indeed, Vertex had only considered physician-scientists for the role. They had big plans for the role — for what they called Vertex 3.0. Although they had become known as the CF company, for years, Leiden told anyone who would listen that he didn't just want to transform one disease: He planned to use the lavish proceeds from those pills to cure CF completely and either cure or defang an Infernal Council of famous ailments: Sickle cell disease, diabetes, muscular dystrophy and pain, among others.

It was as ambitious a plan as a biotech had ever put forward, spanning medical disciplines from hematology to nephrology and technologies from old-fashioned pills to new forms of CRISPR gene editing, and they needed someone with unimpeachable scientific chops to carry it out. If Kewalramani and her team can, they will change the face of medicine: Not just for one rare disease but several, and a few not so rare ones as well. They could also set off the same string of rancorous global debates that have followed Vertex's CF drugs, as the company charged more than what many countries said they could pay. Kewalramani, while striking a less abrasive tone than her predecessor, has pledged to keep the same pricing strategy moving forward.

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May 24, 2021 10:41 AM EDT
R&D, FDA+

FDA may require bispecific antibodies to be compared with mono-specific products

Zachary Brennan
Senior Editor

With about 100 bispecific antibodies either in clinical trials or soon entering, the FDA on Monday updated and finalized its guidance on developing bispecific antibodies, clarifying how it may require clinical trials to compare the bispecifics with an approved monospecific product.

Financing, Deals

Rare disease player that went public in biotech's go-go years merges with specialty pharma in all-stock deal — at a fraction of the debut price

Amber Tong
Senior Editor

Strongbridge Biopharma went public in the summer of 2015, the tail end of a biotech boom on Nasdaq, with a solid pitch. Almost 20 years old at that time, it was to focus on in-licensing, developing and then commercializing treatments for rare diseases that it believes has potential to be franchises in the US and the EU with a tiny salesforce. RA Capital, New Enterprise Associates and Longwood Capital were among its investors.

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R&D

A genetics testing outfit is bailing on its CRO services, choosing instead to refocus around precision drugs

Josh Sullivan
Associate Editor

In the past few months, there's been a string of consolidation in the CRO space, with big players merging together and headline names in contract manufacturing snapping up research teams. But a Texas genetics player is now going the other way, bailing on its CRO offerings to instead focus on precision medicine.

Salt Lake City-based Myriad is selling its Austin contract research lab to North Carolina's Q2 Solutions

May 24, 2021 11:02 AM EDT
Updated 11:24 AM
R&D

A top analyst sees 'inflection point' in the other big vaccine race taking pharma by storm

Jason Mast
Editor

Beneath the Covid-19 vaccine race, there's another vaccine race in full swing, with billions on the line and many of the same leading players edging for first: the race for an RSV vaccine.

Although the seasonal infection can be deadly in young children and the elderly, scientists have failed for decades to come up with a vaccine. An early trial in the 1960s ended in disaster, after it appeared the vaccine actually helped the virus infect cells. Recent advancements, though, have renewed interest in what could be a $10 billion market, bringing in efforts from major developers, including Pfizer, Moderna, J&J, Merck, Sanofi and GlaxoSmithKline.

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Financing

Eliem has put together a $60 million Series B round co-led by RA Capital as it seeks to further advance two lead clinical candidates across four trials. The biotech declined an interview and further declined to comment on an emailed question regarding financing plans, suggesting they may be hunkering down in preparation for an S-1 filing with the SEC.

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May 24, 2021 07:00 AM EDT
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R&D

UPDATED: NGM concedes key failure for their lead NASH drug, closing the door on a pivotal followup — and asking analysts to look past the wreckage into the pipeline

John Carroll
Editor & Founder

Amber Tong
Senior Editor

May 24, 2021 11:21 AM EDT
Manufacturing

Chinese oncology player is the latest to join the North Carolina manufacturing party with 2 new plants in the offing

Josh Sullivan
Associate Editor

While Boston and the Bay Area reign supreme in terms of biotech hotspots, other hubs are having their chance in the sun amid a flurry of cash flooding the industry. North Carolina, for instance, has seen a ton of companies looking to establish a footprint in the state — and now a Shanghai-based oncology player will join the fold.

CARsgen Therapeutics will open two new sites in Raleigh Durham, creating the company's first manufacturing hub that will complement its clinical development site in Houston, the company said Monday.

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Business Model