The CenterWatch Monthly

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Options for AI in Clinical Research Abound, but So Do Challenges, Experts Say

By James Miessler

The burgeoning AI space is bristling with potential for application in clinical trials, from study startup and recruitment to site monitoring visits, protocol simplification and greater operational efficiency overall. But widespread adoption will first require industry to properly evaluate and show the value of solutions in this still-evolving area. The CenterWatch Monthly *reached out to several experts to gather insights on how AI is being used and what value it has for clinical trials.*

Michelle Longmire, CEO and cofounder of Medable, believes AI is poised to have a huge impact on overall drug development times by helping accelerate the entire process, beginning with R&D and extending all the way through to clinical trials. And while the use of AI is just getting started and taking shape in the life sciences industry — Longmire characterizes it as currently being "in the modem phase of the Internet at best" — it has the potential to be a game-changing solution for many of industry's key challenges, she says.

"We were all really chipping away at what we thought were important solutions, and then it's like you discover a new element or a new type of fuel, and suddenly it's kind of a quantum leap opportunity in terms of how you achieve those outcomes," she told *The CenterWatch Monthly*. "We always reference the fact that the number of drugs approved hasn't changed; in fact, it's gone down in recent years. To me, that's a reflection that we haven't really leveraged technology to solve this problem."

One key challenge AI could help solve is the lack of direct trial invitations, an issue that persists as a significant barrier to trial participation and the inclusion of underrepresented groups, according to recent data (*The CenterWatch Monthly*, October 2023).

Whether it's a nurse practitioner at a walk-in clinic or pharmacy or a physician

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Interview: Increased Trial Access is a Global Effort, Technology Provider Says

By James Miessler

Making clinical trials truly representative of the disease populations they target is not an effort confined solely to the U.S. and FDA; it is a global initiative with many considerations that differ by country, culture and regulatory agency. The CenterWatch Monthly interviewed Liz Beatty, cofounder and chief strategy officer at Inato, a tech provider focused on boosting trial inclusivity and access around the world, to discuss thinking from an international perspective on diversity, equity and inclusion (DE&I).

CWM: *What does it mean to look at representative trials through a global lens? Please elaborate on this concept.*

Beatty: We can't tackle representative trials globally until we, as an industry, can agree on what that truly looks like. In the U.S., racial and ethnic disparities are top of mind when we talk representation. However, in other countries, there are privacy, regulatory and cultural differences that need to be accounted for, and in some countries, even collecting this type of data is prohibited. For me, tackling representation at a global scale means recognizing that the scope of our U.S.-based definition of representation doesn't apply equally to all countries. To achieve the goal of providing equitable trial opportunities for all people, we must first take steps to understand what representation really means worldwide. When we've established a firm understanding of which demographics must be included globally to fill gaps in access, we can ensure that the patients in our trials include the people who will ultimately receive the medicine.

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It's Time to Fix the Clinical Research Workforce Crisis

This month's guest columnist, ***Susan Landis, executive director of the Association of Clinical Research Professionals*** *(ACRP), explains why the clinical research workforce is in crisis and how ACRP is on a mission to fix it.*

FDA Commissioner Robert Califf wrote in a Society for Clinical Trials op ed piece earlier this year that "now is the time to fix the evidence generation system." By this, he meant that the clinical research enterprise, which lags far behind discovery science, requires "a major reformation" to efficiently translate invention into implementation.

However, a fundamental pillar of the evidence generation system is the clinical research workforce, which is currently anything but robust. Perhaps the most visible sign of this crisis is the widening gap between supply and demand for competent staff, but underpinning this is a perfect storm of complex issues that ultimately jeopardizes the engine of drug and device development.

In 2022, there were 1 million more vacancies for clinical research professionals than available candidates, according to the Society for Clinical Research Sites, and the resignation rate was 60 percent higher than in 2020. A fierce "war for talent" with overt poaching and unsustainable levels of turnover has ensued, which is likely to intensify as demand for clinical trials ramps up, alongside increasing appetite for decentralized trials with their specialized skillsets. Unless the workforce crisis is fixed, ambitious research missions like the Biden administration's Cancer Moonshot, which aims to cut the cancer death rate in half by 2047, could fall at the first hurdle.

In the interests of a robust, research-ready workforce, ACRP is on a mission to revolutionize clinical research as a career.

At the heart of the workforce crisis is the fact that clinical research professionals are not just doing a job, they are pursuing a career. But investment, support and infrastructure for this career are woefully lacking. Even though drug development is one of the world's most regulated industries, matched only by the commercial nuclear industry, those who conduct clinical research have no professional identity, no standardized qualifications and no visible pathway into their career. Clinical research is not recognized as a career by the U.S. Bureau of Labor Statistics and is poorly represented in STEM curricula. Most clinical research professionals find their way into the career by chance.

This situation contrasts starkly with other careers that also critically depend on skills and competencies. Airline pilots, for example, who are entrusted with the safety and security of passengers and crew and with efficient compliance with operating procedures, don't stumble or wander into their career; they choose it. From the start, they understand the career pathway, undergo systematic training in all the fundamentals and gain explicit qualifications recognized by their employers.

Site-based clinical research professionals are the pilots of clinical trials, carrying a heavy responsibility for the safety and well-being of participants, for strict adherence to Good Clinical Practice, trial protocols and ethical principles, and ultimately for the lives of study participants. Management of the global COVID-19 crisis was fundamentally dependent on these professionals operationalizing the development of vaccines and treatments at record speed. So, in the world's most regulated industry, how is it acceptable that those in the cockpit have no mandatory qualifica-

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Billing, Informed Consent, Performance Assessment Among Top Subjects at MAGI

WCG's recent virtual conference, MAGI@home, featured expert commentary on a number of important clinical research topics, including clinical operations, quality and regulatory issues, budgets ad contracts, and billing compliance. The following are some insights from conference presenters.

Billing Compliance

Attendees learned important pointers for staying compliant with research billing regulations, including the federal Anti-Kickback Statute, the Stark Law and the False Claims Act, from Amanda Miller, manager of quality and development for WCG, and Carly Tucker, corporate compliance manager for Tufts Medicine.

They encouraged sites to take a number of measures to support their billing compliance efforts and protect themselves from violations, including:

- Train research administration staff to be aware of the regulations and recognize problematic language in draft budgets, contracts and informed consent forms, and set up communication channels for these employees;
- Develop billing and claims processing controls that establish a documented claims review process, ensure most if not all trials opened have a coverage analysis on file, and provide basic billing compliance training to all staff involved;
- Align coverage analyses, budgets, contracts and informed consent forms with each other, including in any software systems, and determine if your institution uses a contracts management system; and
- Create a system for assessing the financial relationships of physicians and make a contracting process.

Obtaining Informed Consent

Jamie Lucey, senior director of quality and systems for Circuit Clinical, and Jennifer Peterson, director and head of clinical quality for M3 Wake Research, walked attendees through the FDA's recently released guidance on regulatory expectations for informed consent roles and procedures.

In addition, they went in depth on ways to nail the informed consent process and address challenges that may come up, including special considerations for certain patients. On this topic, Lucey discussed things to think about when consenting non-English speakers, low literacy/numeracy patients, patients with varying capacity to consent and patients with physical and/or sensory disabilities.

For example, non-English-speaking patients should not be excluded unless the protocol directs sites to do so, she advised. In dealing with these patients, it's important to accommodate them by sharing information and explaining medical terms in a language and at a level they can understand. As part of this, the IRB should review and approve procedures that ensure translations will be provided by a qualified individual or provider and interpreter support will be made available. Lucey noted that it is inappropriate for family members to provide interpretations.

For low literacy/numeracy patients, modifications to the informed consent process may be necessary. In some circumstances, a shortened version of the informed consent form and a witness can suffice, while patients who cannot write can use their mark to sign and date the consent form rather than their name. With these patients, it's important to document the process used and note the actual date of consent in the patient source.

Key Performance Indicators for Site-Sponsor-CRO Relationships

Dana Austin, executive director of The Global Chamber and clinical research consultant, and Kristen Ballesteros, associate director of feasibility strategy for Thermo Fisher Scientific, discussed the growing importance of key performance indicators (KPI) in optimizing trial operations, especially as they pertain to relationships between sites, sponsors and CROs.

A number of KPIs can be used to evaluate how sites, sponsors and CROs work together during a trial, including:

- Sponsor and CRO oversight;
- The schedule and planned time for first patient in;
- Trial timelines;
- Quality of data;
- Protocol compliance;
- Budget adherence;
- Communication and timely resolution of issues;
- Site performance metrics;
- Staffing concerns and training needs;
- Regular/ongoing meetings;
- Regulatory compliance; and
- Data management (adverse event reporting in a timely fashion, patient safety and efficacy, audit readiness, contractual and legal compliance, stakeholder satisfaction and risk management).

"It's important to tailor KPIs to the specific needs and goals of the clinical trial and the roles of the clinical site, sponsor and CRO," Austin said. "Regular monitoring and assessment of these KPIs can help ensure that all parties involved work together effectively and contribute to the overall success of the clinical trial."

New Site Owner Learns Hard Lessons About Recruitment

Recruitment has long been known to be one of the most difficult parts of clinical research and can be the biggest hurdle for sites just getting into trials. In this new CenterWatch Monthly *column on sites' startup successes,* ***Ruby Hussain, owner and principal investigator of Prime Global Research****, a site based in The Bronx, N.Y., recalls how her site overcame major recruitment challenges they encountered despite being in one of the most densely populated areas of the U.S.*

As a new research site years ago, we faced many challenges when we landed our first trial. Being a new site took much work. There are many challenges that a new site faces, including budgeting, regulatory paperwork, recruitment/retention and staffing, among many others. We soon learned that getting selected as a potential site was the easy part for us, while recruitment was the hardest, even though we are in a very densely populated area of New York.

We started our first trial in 2019. I believed that owning my practice would be sufficient to help me recruit the patients I was looking for, but that was not the case. I recall my staff approaching patients and quickly being given the cold shoulder or a straight out "NO." Most patients did not know what clinical research was, while others did not care to know, so many were hesitant to participate. I recall reaching out to the local physicians in our area and driving around for hours, distributing cards and flyers to the offices in hopes we would get referrals. The process was more complicated than I had anticipated. During this process, I realized two crucial things: one, I must believe in what I am doing, believe in the clinical trial, and know the protocol as if I wrote it. Two, I needed to connect with the people in my community and get to know them and their fears.

First, I spent hours educating myself on the protocol so I understood its science. I then connected with my patients by speaking to them about the trial. I explained everything I knew in addition to the information they read in the consent form in a way they would understand. I began to get directly involved in the recruitment process and educate our community on clinical research and how it can impact their lives. We educated our patients on how clinical trials are crucial in advancing medical research and improving their lives or the lives of loved ones in many ways. We taught them that they are part of history by being part of a cure.

We decided to use a different strategy for recruitment, given our area, taking a straightforward approach. We began distributing flyers to the community members in their settings, such as churches, pharmacies and grocery stores. We got more involved with our community. We learned the barriers that lie within our community in terms of recruitment, including language, education status, family structure, time, and a basic fear of being treated like a guinea pig. Steadily, we started getting past the walls between us and successful recruitment.

As a principal investigator, I spoke to each potential patient and talked them through their fears of the unknown when participating in research. Taking the extra few minutes to help them understand the concept of research and the purpose of the study overall helped me develop long-term relationships and trust in our community. Five years later, we have an established presence and trust within our community. We no longer struggle with the recruitment or retention of patients.

No two trials are alike, and each clinical trial will have its unique challenges, whether it's patient recruitment or the complexity of the protocol. Sometimes, you must step back and think outside the box to help overcome the obstacles.

AI

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at Johns Hopkins, AI holds the potential to help healthcare providers match their patients to trials earlier, Longmire says. This could come, for instance, in the form of an AI-generated visit summary that points patients toward possible trials following their appointments.

In this same vein, AI also has the potential to not just connect patients to trials by way of automation, but to help time-starved providers inform patients about meaningful research and become well-versed on trial opportunities themselves.

"One of the big barriers is the time the practitioners have to be educated themselves and educate the patient on the clinical trial opportunity," she says. "I think that's where, especially around large language models, being able to evaluate, match and actually communicate relevant information to patients is a potentially big solution and big opportunity."

Another exciting application is in study startup, one of the parts of clinical research in which components remain highly manual endeavors, she says.

The site feasibility process, for instance, could be streamlined through automation to make it less stressful and taxing for sites and more insightful for sponsors, while AI solutions could be utilized to slash the overall time it takes to identify and activate patients in trials. In particular, AI could help sponsors identify well-suited sites and understand the true availability of those sites' patients, Longmire says.

Sponsors in search of "needle-in-the-haystack" patients could comb through real-world data using AI to find patients that meet certain eligibility criteria and understand these patients' standard of care, adds Rohit Nambisan, CEO and cofounder of Lokavant, a clinical intelligence company that helps improve trial execution through analytics. AI can be employed in similar ways to analyze enrollment data and provide sites with feedback on their diversity efforts and was used for this very purpose in Moderna's COVID-19 vaccine trials.

"If you're using multiple sources of data, for example, you're working with five different community centers that are providing you access to their EMR and EHR data in a deidentified manner, you need to harmonize this data and actually understand where the biggest bang for your buck is," Nambisan says. "Frankly, it's very hard to do that in manual ways. I think this is a place where large language models or generative AI models can be used to … master that data in a manner that you can compare apples to apples amongst that dataset."

AI could also become a powerful, established tool for sponsors to glean big picture insights from study monitor visit reports, particularly for trials that have many reports, monitors and sites, Nambisan says. Large language models are already being used by sponsors in this way.

Manually, it's a massive endeavor to go through all monitoring visit reports, identify issues and understand their severity and frequency. But using automation, an AI solution can be fed monitoring visit reports for a trial on a regular basis and not just summarize them for sites and sponsors, but also identify trends and highlight impactful findings that can be put into action. This could even enable valuable predictive capabilities for sponsors and sites.

"AI is excellent at automating mundane, repetitive tasks, and clinical trials are full of them," adds Brad Sibbald, vice president of Kelly Science & Clinical. "Leveraging AI to manage time-consuming manual tasks like data entry, scheduling and monitoring allows clinical staff to spend more time on patient care and in-depth data analysis. It also optimizes clinical resources, ensures timelines are met and reduces errors. I'm a big believer in people-first AI tools that allow workers to focus on more enjoyable and impactful tasks."

AI also holds great potential for going through complex, massive sets of data and delivering a greater understanding of responses to investigational products, he says, and has been able to pick up on asymptomatic responses to treatments that would have been missed by the human eye.

But with AI's potential and growth — Sibbald estimates there are approximately 10,000 AI and automation job openings on any given day in the U.S. as demand rises for specialized roles — a hurdle to its widespread adoption emerges, he says: the need for companies to both develop existing employees and connect with new talent.

Sibbald advises companies to turn to specialized staffing firms on this front and address shifting job responsibilities by establishing ongoing training and upskilling programs as AI opens employees up to other duties. Being prepared to revise job positions and in some cases reassign staff members entirely will also be critical, he says.

"As skills and responsibilities evolve alongside AI, employers must consider that certain job descriptions will change and proactively develop plans to redesign those roles. In addition, some roles may disappear entirely, and organizations should think now about how to best redeploy talent currently in those roles," Sibbald says.

Moving forward, industry stakeholders will also need to ensure that they can properly prove and demonstrate the value of AI use in clinical research or adoption will not happen at large scale and AI will not move out of these very beginning stages, Longmire and Nambisan say.

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AI

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Though it holds tremendous promise, AI will take longer to catch on and become widely accepted in the clinical research industry due to the heavily-regulated space stakeholders navigate and the involvement of human participants, Nambisan says. This means that industry should ensure AI models truly function as intended, cutting their teeth on more administrative, repetitive tasks that won't impact patient safety before they're moved into the risk-prone environment of trials, he says.

"Once we do that and quantify exactly what the value delivered is on those administrative tasks and get people more used to working with such models in software, then I think we can start to move into more of a mature cycle, using them on specific use cases where highly specialized knowledge is required and indicating how valuable they can be," he said.

"This is an evidence-based industry, so I think what's important is we are willing to incur some tax, not around safety or efficacy of drugs, but around being able to do something new," Longmire adds.

"In some cases, it might feel harder, but that's a really important part of the curve for progress," she continues. "I think it's really important we get a lot of learnings in and continue to prove the value [of AI]. We won't get where we want to get if we're not realistic about what it's going to take, if people aren't willing to see it through."

Beatty

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CWM: *There is a lot of talk in the DE&I conversation about connecting with patients/potential participants in their communities and "bringing the trial to the patient." How does this idea apply to global trials?*

Beatty: Healthcare systems and how people receive care varies globally, but the appeal of having the option to receive innovative treatment from a trusted care team remains universal. Recognizing that access to care and inclusion in clinical trials have unique challenges country to country is crucial for sponsors looking to diversify enrollment globally. The individuals best suited to navigate this successfully are local community researchers. They have the ability to bridge cultural gaps and have established local connections that foster patient engagement. When we give these trusted local research teams the opportunity to conduct a trial, we're empowering them to engage their community and enroll underrepresented patients, unlocking global patient access and driving more inclusive and impactful trials.

CWM: *For trials in indications that have very few participant prospects and probably must spread out across the globe, such as rare/ultra rare diseases and oncology trials with tough eligibility criteria, what considerations come into play for DE&I?*

Beatty: For complex studies like rare diseases or oncology trials, recruiting patients is already a significant challenge; it can take years just to enroll in a trial. Requiring specific subpopulations to participate has the potential to stall these studies even further, slowing down the development of potentially life-saving medications for the people who need them most. Additionally, an increased focus on enrolling specific subpopulations further compounds the trial's complexity, elevating costs for sponsors and increasing costs for payers and patients once that medicine becomes commercially available. The answer is clear: We can't achieve diversity in complex trials by doing what we've always done. The current clinical trial model doesn't allow for a solution that benefits patients across the board. Instead, we should look at ways to flip the traditional process, employing innovative alternatives, such as exploring more avenues for implementing real-world data and investing in emerging tech solutions with the ability to disrupt the industry and pave the way for reliably representative trials, even in the most complex disease areas.

"We can't achieve diversity in complex trials by doing what we've always done. The current clinical trial model doesn't allow for a solution that benefits patients across the board."

Liz Beatty, cofounder and chief strategy officer, Inato

CWM: *From an FDA regulatory perspective, what is different about diversity in global trials compared to trials conducted locally and in the U.S.? How do expectations differ? I assume there are also approaches that differ in effectiveness in global trials compared to smaller-scale single-country trials.*

Beatty: Discussions around diversity and representation in the U.S. are largely defined by the unique historical dispari-

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ties that have affected a range of demographics in our country. Unique being the keyword here — every other country is also striving to address unique disparities, so to enroll representative trials globally, we must broaden our definition of diversity to include all underrepresented populations worldwide. At the end of the day, the FDA is looking for good science that has evaluated the safety and efficacy of an investigational medicine across all the subpopulations that will receive that medicine once approved. We rely on global data to ensure this is possible, so from a regulatory perspective, these trials must meet the needs of health authorities around the world to reach the demographics that make up our global population. This means that while representative populations may look different country to country, it is the end result of broadly inclusive research that matters.

CWM: *With the "global lens" in mind, can you comment on the diversity action plan the FDA is expecting of sponsors and how these action plans are being handled in international trials?*

Beatty: For the FDA, the patients' demographic is a crucial factor when building diverse action plans; the drug must be proven safe for patients of all biological backgrounds. Sponsors looking to meet diversity requirements based on specific ethnic and racial subpopulations should see expanding globally as an opportunity to effectively target populations that are underrepresented in research or disproportionately affected by a disease. For example, Asian populations are disproportionately affected by hepatitis B. By planning for the trial to recruit in Asian countries where hepatitis B is prevalent, sponsors can more reliably identify the most relevant patient populations. Partnering with research sites across the globe can improve the sponsors' ability to meet the FDA's representation requirements, with an additional benefit of expanding the global accessibility of these trials.

CWM: *Overall, where are we globally in efforts to make trials adequately diverse and representative of their corresponding disease populations?*

Beatty: It's clear that the U.S. is not alone in its goal to improve representation in trials. Regulatory bodies in other countries, Canada and Australia, for example, are putting out requirements similar to the FDA's to ensure that the patients enrolling in clinical research represent those affected by the disease. However, these are not harmonized efforts. Country by country, we're striving for different targets using unique categorizations that make the analysis of this data more complex. The bottom line is, we're just getting started addressing this and, so far, our approach isn't working. If we continue to individually interpret the meaning of representative data, we risk losing sight of the bigger picture: developing safe and effective treatments for all people across the globe. Until the industry can work together to determine what trial representation looks like globally for clinical trials, we won't be able to reliably conduct trials that truly accomplish representative research.

Viewpoint

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tions or explicit career path?

In the interests of a robust, research-ready workforce, ACRP is on a mission to revolutionize clinical research as a career. Partnering with an industrywide consortium, we are mapping out a clear career pathway, underpinned by a strong career identity. As a prerequisite, we are generating baseline data on the size, demographics and defining features of the global clinical research workforce. We're also building and piloting a curriculum for clinical research within high schools.

Our second objective is to change how clinical research professionals are hired. Despite the increasing number of academic programs for aspiring researchers in this field, an archaic hiring preference for two to four years of experience blocks many promising candidates. This has created a Catch-22 situation where experience is needed to get a job, but a job is needed to gain experience. Leveraging the competency framework developed by the Joint Task Force for Clinical Trial Competency, we're aiming to formulate a competency-based approach to hiring entry-level clinical research professionals and to drive industrywide adoption.

Finally, although there is no shortage of educational and training courses, they are often not explicitly aligned with employers' prerequisites. To open doors into the career, we're looking to harmonize educational pathways with the needs of employers and develop tools for learners and their managers to track the acquisition of skills. We're also aiming to create a standardized internship model.

Ultimately, we aspire to elevate awareness and embed infrastructure to ensure a career in clinical research is universally recognized, professionalized and fulfilling. The evidence generation system and those who turn its wheels — recently referred to by the FDA as the "beating heart of the industry" — deserve nothing less. However, initiatives like ours can only make a difference if they are universally championed. Essentially, this calls for a collective mindset shift and multidisciplinary, enterprisewide cultural change.

The opinions expressed here are those of the author and do not necessarily reflect the views of The CenterWatch Monthly.

Study Lead Opportunities

CenterWatch analyzes data in its drug intelligence database to provide advance notice of clinical trials expected to enter the next phase of clinical development soon. Contact information is provided for follow-up. **Sponsors/CROs:** to list an upcoming trial here, contact Leslie Ramsey, 703.538.7661, lramsey@wcgclinical.com.

Company name	Drug name	Indication
phase 1		
Biomea Fusion	BMF-500	Relapsed/refractory acute leukemia
Cerevance	CVN293	Amyotrophic lateral sclerosis and Alzheimer's disease
Disc Medicine	DISC-3405	Polycythemia vera
Drug Farm	DF-003	Cardiorenal diseases and ROSAH syndrome
Entrada Therapeutics	ENTR-601-44	Duchenne muscular dystrophy in patients who are exon 44-skipping amenable
Flare Therapeutics	FX-909	Advanced solid malignancies, including advanced urothelial carcinoma
Gain Therapeutics	GT-02287	Parkinson's disease
HanAll BioPharma Daewoong Pharmaceutical NurrOn Pharmaceuticals	HL192	Parkinson's disease
HanchorBio	HCB101	Advanced solid tumors or relapsed/refractory non-Hodgkin lymphoma
Kura Oncology	KO-2806	Advanced solid tumors
Radionetics Oncology	68Ga-R8760	Adrenocortical carcinoma
SystImmune	BL-B01D1	Metastatic/unresectable non-small cell lung cancer
Vir Biotechnology	VIR-1388 vaccine	HIV prevention
phase 1a/1b		
ImmunoGenesis	IMGS-001	Relapsed/refractory advanced solid tumors
Numab Therapeutics	NM26	Moderate-to-severe atopic dermatitis
Synthekine	STK-012	Solid tumors
phase 1/1b		
Revolution Medicines	RMC-9805	Cancers with the KRASG12D mutation
phase 1b		
Amplifier Therapeutics	ATX-304	Cardiometabolic diseases
ClearB Therapeutics	CLB-3000	Chronic hepatitis B

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Study Lead Opportunities continued from page 8

Company name	Drug name	Indication
phase 1b (continued)		
Starton Therapeutics	Low-dose lenalidomide (STAR-LLD)	Multiple myeloma
Tenaya Therapeutics	TN-201 gene therapy	MYBPC3-associated hypertrophic cardiomyopathy
Virion Therapeutics Ocean Biomedical	VRON-0200	Chronic hepatitis B
phase 1b/2		
Armata Pharmaceuticals	AP-SA02	Staphylococcus aureus bacteremia
CytoAgents	CTO1681	Cytokine release syndrome
GenFleet Therapeutics	GFH009	Relapsed/refractory peripheral T-cell lymphomas
Gracell Biotechnologies	GC012F	Relapsed/refractory multiple myeloma
Moleculin Biotech	Annamycin plus Cytarabine (Ara-C)	Acute myeloid leukemia
NervGen Pharma	NVG-291	Spinal cord injury
Protara Therapeutics	TARA-002 intravesical instillation	High-grade nonmuscle invasive bladder cancer patients with Bacillus Calmette-Guérin (BCG)-naïve and BCG-unresponsive carcinoma in situ
phase 1/2		
AviadoBio	AVB-101 gene therapy	Frontotemporal dementia with GRN mutations
BioInvent	BI-1808	Advanced malignancies
Bolt Biotherapeutics	BDC-3042	Metastatic/unresectable triple-negative breast cancer, colorectal cancer, clear-cell renal cell carcinoma, head and neck cancer, non-small cell lung cancer, and ovarian cancer
Cantex Pharmaceuticals	Azeliragon	Refractory metastatic pancreatic cancer
Clarity Pharmaceuticals	64Cu/67Cu SAR-Bombesin	Metastatic castration- resistant prostate cancer
ESSA Pharma	Masofaniten plus enzalutamide	Metastatic castration-resistant prostate cancer
ImmPACT Bio	IMPT-314	Relapsed/refractory aggressive B-cell lymphoma
IMUNON	IMNN-001 plus bevacizumab	Advanced ovarian cancer
LinKinVax	CD40HVac HPV vaccine	HPV-positive oropharyngeal cancer
MBX Biosciences	MBX 1416	Post-bariatric hypoglycemia
Oncternal Therapeutics	ONCT-534	Metastatic castration-resistant prostate cancer
Scorpion Therapeutics Pierre Fabre Laboratories	STX-721	Locally advanced/ metastatic non-small cell lung cancer with EGFR ex20ins mutations
Smart Immune	SMART101 allogeneic cell therapy	Acute leukemia and myelodysplasia syndrome in adults

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Company name	Drug name	Indication
phase 2a		
Agomab Therapeutics	AGMB-129	Fibrostenosing Crohn's disease
Inmagene Biopharmaceuticals	IMG-007	Alopecia areata
NeuroBo Pharmaceuticals	DA-1241	Nonalcoholic steatohepatitis
Zucara Therapeutics	ZT-01	Preventing nighttime hypoglycemia in type 1 diabetics
phase 2		
Cantex Pharmacueticals	Azeliragon	Newly diagnosed unmethylated glioblastoma
Carrick Therapeutics	Samuraciclib plus elacestrant	CDK4/6i resistant HR+ HER2- metastatic breast cancer
Cyrano Therapeutics	CYR-064	Post-viral hyposmia
ImmunoForge	Froniglutide	Dermatomyositis/polymyositis
IN8bio	INB-400	Newly diagnosed glioblastoma multiforme
Kallyope	K-757 and K-833	Obesity and type 2 diabetes
NMD Pharma	NMD670	Spinal muscular atrophy
Vedanta Biosciences	VE202	Ulcerative colitis
phase 2b/3		
Cantex Pharmaceuticals	Azeliragon	Life-threatening complications in patients hospitalized for COVID-19
phase 3		
Apnimed	AD109 (aroxybutynin/ atomoxetine)	Obstructive sleep apnea
Ascentage Pharma	Olverembatinib	Philadelphia chromosome-positive acute lymphoblastic leukemia
Boehringer Ingelheim Zealand Pharma	Survodutide	Treatment of obese or overweight people with comorbidities but not type 2 diabetes
Boehringer Ingelheim Zealand Pharma	Survodutide	Treatment of obese or overweight people with comorbidities, including type 2 diabetes
Boehringer Ingelheim Zealand Pharma	Survodutide	Treatment of obese or overweight people with cardiovascular disease or chronic kidney disease
BriaCell Therapeutics	Bria-IMT	Advanced metastatic breast cancer
Foresee Pharmaceuticals	Leuprolide	Central precocious puberty
YS Biopharma	PIKA Rabies Vaccine	Rabies
phase 3b/4		
CymaBay Therapeutics	Seladelpar	Cirrhosis due to primary biliary cholangitis

FDA Actions

The following is a sampling of FDA regulatory actions taken during the previous month, compiled from CenterWatch and third-party sources, including the FDA and company press releases. For more information on FDA approvals, visit centerwatch.com/fda-approved-drugs.

Company name	Drug name	Indication	FDA action
Akeso	AK117 plus azacitidine	Newly diagnosed higher-risk myelodysplastic syndromes	IND approved
Aligos Therapeutics	ALG-055009	Nonalcoholic steatohepatitis	IND approved
AltruBio	ALTB-268	Ulcerative colitis	IND approved
Biomea Fusion	BMF-219	Type 1 diabetes in adults	IND approved
Cabaletta Bio	CABA-201	Systemic sclerosis	IND approved
ContraFect	CF-370	Hospital-acquired bacterial pneumonia and ventilator-associated bacterial pneumonia	IND approved
Hepagene Therapeutics	HPG7233	Nonalcoholic steatohepatitis and dyslipidemia	IND approved
Intellia Therapeutics	NTLA-2001 gene therapy	Transthyretin (ATTR) amyloidosis with cardiomyopathy	IND approved
Jasper Therapeutics	Briquilimab	Chronic spontaneous urticaria	IND approved
Krystal Biotech	KB408	Alpha-1 antitrypsin deficiency	IND approved
LAPIX Therapeutics	LPX-TI641	Multiple sclerosis	IND approved
MAIA Biotechnology	THIO	Advanced non-small cell lung cancer	IND approved
Oscotec ADEL	ADEL-Y01	Alzheimer's disease	IND approved
Osmol Therapeutics	OSM-0205	Chemotherapy-induced peripheral neuropathy	IND approved
Pulmatrix	PUR3100 (dihydroergotamine mesylate inhalation powder)	Acute migraine	IND approved
Terns Pharmaceuticals	TERN-701	Chronic myeloid leukemia	IND approved
Y-mAbs Therapeutics	CD38-SADA	Relapsed/refractory non-Hodgkin lymphoma	IND approved
Alzamend Neuro	AL001	Bipolar disorder type 1	IND approved
Transcenta	Osemitamab (TST001) plus nivolumab and chemotherapy	First-line gastric/gastroesophageal cancer	IND approved
Creative Medical Technology	StemSpine using AlloStem (CELZ-201-DDT)	Chronic lower back pain	IND approved

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FDA Actions continued from page 11

Company name	Drug name	Indication	FDA action
Orchestra BioMed	BackBeat CNT (atrioventricular interval modulation therapy)	Hypertensive patients indicated for a dual-chamber cardiac pacemaker	IDE approved
Protembis	ProtEmbo Cerebral Embolic Protection System	Transcatheter aortic valve replacement	IDE approved
ReGelTec	HYDRAFIL System	Chronic low back pain due to degenerative disc disease	IDE approved
Ardelyx	Xphozah (tenapanor)	Hyperphosphatemia in adults with chronic kidney disease on dialysis	Approved
Amicus Therapeutics	Pombiliti (cipaglucosidase alfa-atga) plus Opfolda (miglustat)	Late-onset Pompe disease	Approved
Apellis Pharmaceuticals	Empaveli (pegcetacoplan) Injector	Paroxysmal nocturnal hemoglobinuria	Approved
Fabre-Kramer Pharmaceuticals	Exxua (gepirone hydrochloride extended release tablets)	Major depressive disorder in adults	Approved

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FDA Actions continued from page 12

Company name	Drug name	Indication	FDA action
GlaxoSmithKline	Ojjaara (momelotinib)	Intermediate- or high-risk myelofibrosis patients with anemia	Approved
Novo Nordisk	Rivfloza (nedosiran) injection	Treatment of patients age 9 years or older with primary hyperoxaluria type 1 and relatively preserved kidney function	Approved
Orasis Pharmaceuticals	Qlosi (pilocarpine hydrochloride ophthalmic solution) 0.4%	Presbyopia	Approved
Pfizer	Velsipity (etrasimod)	Moderate-to-severe ulcerative colitis	Approved
UCB	Bimzelx (bimekizumab-bkzx)	Moderate-to-severe plaque psoriasis	Approved
UCB	Zilbrysq (zilucoplan)	AChR antibody-positive generalized myasthenia gravis	Approved
Viatris Ocuphire Pharma	Ryzumvi (phentolamine ophthalmic solution) 0.75%	Pharmacologically induced mydriasis	Approved
Redhill Biopharma	Talicia (amoxicillin, rifabutin and omeprazole)	H. pylori infection	Approved for new dosing regimen
Boehringer Ingelheim Eli Lilly	Jardiance (empagliflozin)	Chronic kidney disease in adults	Approved for new indication
Pfizer	Braftovi (encorafenib) plus Mektovi (binimetinib)	Metastatic non-small cell lung cancer with a BRAF V600E mutation	Approved for new indication
Merck	Keytruda (pembrolizumab)	Resectable (tumors greater than or equal to 4 centimeters or node positive) non-small cell lung cancer	Approved for expanded indication
Novartis	Cosentyx (secukinumab) intravenous	Ankylosing spondylitis, psoriatic arthritis and non-radiographic axial spondyloarthritis	Approved for new formulation
Saptalis Pharmaceuticals	Likmez (metronidazole oral suspension)	Bacterial infections	Approved for new formulation
Arcutis Biotherapeutics	Zoryve (roflumilast) cream	Plaque psoriasis in children age 6 to 11 years	Approved for expanded age indication
Laminate Medical Technologies	VasQ External Vascular Support	Creating arteriovenous fistulas for dialysis access	Approved

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