The CenterWatch Monthly

In This Issue



January 2024 A CenterWatch Publication Volume 31, Issue 01

The Year Ahead

# Experts Predict Big Things for Data and Cross-Industry Collaboration in 2024

*Experts anticipate the new year will prove to be an impactful one for data and technology in R&D and clinical trials as companies seek to leverage big data in practical ways, simplify end-to-end data flow for sites, build single platforms for managing data and work together to drive the AI revolution forward. The following are some insights for 2024 from experts at Lokavant and Veeva Systems.*

**Rohit Nambisan**
CEO and Cofounder
Lokavant

Data-sharing and cross-industry collaboration will power the AI revolution in clinical research in 2024. No one company has enough data to drive accurate productions around a single disease or use case, which is why cross-industry collaboration like we witnessed during the pandemic will be reinvigorated. During COVID, pharmaceutical companies, clinicians, researchers, technology companies and regulators worked together in harmony, so we know it can be done.

A current example of how this can work is the MELODY trial, which is using federated learning, a data-sharing model that protects companies' proprietary information while still sharing important research data, to provide much-needed, high-quality protein data to help AI/ML models design proteins faster. Protein drug development is notoriously long, arduous and costly. But in the MELODY trial, the contributing organizations can use AI to adopt generative biology and are experiencing greater efficiency than any individual organization could alone.

Without this level of collaboration, AI won't provide tangible return on investment and and adoption will slow to a trickle.

**Andreas Matern**
Executive Vice President of Product
Lokavant

Personalized medicine development will continue its fast pace in 2024, which

see **Data** on page 4

The Year Ahead

# DCTs, Digital Therapeutics to Make Further Headway in 2024, Say Experts

*Decentralized trial (DCT) methods will continue to evolve in 2024 as a group effort led by the Tufts Center for the Study of Drug Development (CSDD) to gather/utilize DCT data for informing future protocol designs aims to deliver its first dataset in the first quarter. By the end of 2024, decentralization will have established itself as the new gold standard for trials, experts predict.*

*In addition, experts believe that as digital therapeutics (DTx) continue their transition away from prescription models in favor of a straight-to-consumer approach, clinical trials will still be necessary for these products as devicemakers confirm their claims and value, though FDA approvals won't.*

**Pamela Tenaerts**
Chief Scientific Officer
Medable

By the end of 2024, the life sciences industry will replace hypothetical surveys about the impact of decentralized methodologies in clinical research with hard evidence. Tufts CSDD's precompetitive consortium of 50-plus companies will collate metrics to understand how decentralization impacts protocol performance, such as cycle times, patient recruitment and retention rates. The project was funded by the Reagan-Udall Foundation for the FDA and Medable and will be a multiyear effort.

see **DCTs** on page 5

# Using Collaboration and AI to Repurpose Already-Approved Medications for New Indications

***Steve Smith**, president of patient advocacy at WCG shares insights into the controversial topic of repurposing drugs to treat new conditions.*

At first, the idea of repurposing a drug seems so logical. A reasonable person could be forgiven for thinking this is already common practice. Since safety and efficacy have been shown in order to gain FDA approval to use a drug for a specific disease, that logic goes, wouldn't researchers check to see what other diseases might be treated using such a drug?

No, they wouldn't, and they don't. Lives are lost because we don't do enough with the information we already have.

There are multiple reasons for this. All of them make sense when we consider the cost of clinical trials. Many of the indications that might be additional matches for such an approved drug are rare. Since additional clinical trials for subsequent indications cost a lot, that discourages further innovation.

Also, if there were to be an adverse event within a new trial, the existing approved drug might be at risk. There are more reasons. Among them are lack of information about the scientific and genetic characteristics of diseases and how they line up with the mechanisms of action of the approved drug. Often, disease characteristics are buried deep in written journal articles. How is a researcher to know all the possibilities?

Most reasons for not repurposing drugs, such as economic or political hurdles, have solutions. Why not, for example, create incentives for drug developers to repurpose drugs for rare indications? Wouldn't this address the unwillingness of drug developers to risk such enormous amounts? Could we not reduce the tax burden or extend their period of post-approval exclusivity to help them recoup return on investment?

Yes, some think. Such incentives have worked to elevate rare disease research to a mainstream form of drug development and lives have been saved. Drug repurposing has not been supported by similar legislation.

**"Most reasons for not repurposing drugs, such as economic or political hurdles, have solutions. Why not, for example, create incentives for drug developers to repurpose drugs for rare indications?"**

—Steve Smith, President of Patient Advocacy, WCG

Proposals for new repurposing legislation have been shut down almost unnoticed in the heated legislative battles regarding high drug prices, perceived pharmaceutical industry excesses, and the need to appeal to a voting public with easier to understand positions. The nuance of drug repurposing incentives seems too much for legislators to understand, or to get across, in the time they feel they have available to focus on it.

What is certain is that drug repurposing works. Individual cases that dramatically demonstrate this can be found going back years. About 18 years ago, a mother, Dorelia Rivera, saw a drug repurposing opportunity save her baby daughter's life. A serious auto-inflammatory disease was stealing her daughter's eyesight, causing damage in multiple systems, alarming pain, and threatening her life. Rivera remembers this early discussion, "the doctor … said … 'I have no idea how to treat it, but I have read that she won't live to see ten years old.'"

After that, teaming up with the NIH and two pharmaceutical companies, an already-approved drug for rheumatoid arthritis was repurposed in a research study done for this little girl. It worked, and today this lucky girl is in college thanks to the initiative to repurpose the drug. Fortunately for her, someone thought of that drug as a potential match for her disease.

There are numerous cases with such good outcomes, but they happen against a strong current of obstacles. Many people die or lose quality of life because no one learns in time that there is a drug that could be repurposed for them, or no one will fund the research. It is just too "one-off" stakeholders might think. In many cases, researchers don't know where to start identifying a drug to repurpose.

Recently, however, a small but important surge of success in drug repurposing that is saving lives and demonstrating a model that should be emulated, scaled up, and mainstreamed is happening at the University of Pennsylvania Medical Center's Centers for Orphan Diseases and Cytokine Storm Research led by David Fajgenbaum. Fajgenbaum is well known in rare disease research for having done such repurposing on himself,

see **Guest Column** on page 6

# Reflecting on MAGI's Impact on Industry Ahead of New Orleans Conference

*With this year's fully virtual MAGI@home conference delivering high-energy insights and receiving a strong reception from attendees, 2024's in-person MAGI conference in New Orleans on April 14-17 is sure to generate more of the same. The following is select coverage from the WCG Talks Trials podcast, which discussed MAGI's success over the years.*

To Geoffrey Schick, WCG's director of strategic site partnerships, the conference has demonstrated "durability and the ability to be a long-term solution" for the strong and loyal attendee base of clinical research professionals it has built. But more important than that, it has fostered critical engagement between sponsors, CROs, sites and regulators that's led to positive changes in clinical research.

Take, for example, industry's attention to billing compliance for Medicare coverage analysis in the late 2000s, a time when that activity was still relatively fresh. While there were a few consultants capable of training people on it, sites were just starting to adopt it and still working to get sponsors to regularly foot the bill, Schick said.

But this support sites once struggled to get from sponsors is now widely considered common practice, in large part due to the topic being featured and discussed at MAGI conferences over the years.

"Through a lot of the work at MAGI in terms of … sharing of ideas and having conversations between sites and sponsors, that has become more the norm now, where there's very few sponsors that don't expect to see in their study startup fees some component of a startup fee for the … Medicare coverage analysis," he said during the podcast. "It was sites and sponsors coming together and understanding the need for the value that it brought in and how it's become known."

"When you get [hundreds of] clinical research leaders … together to talk about topics such as virtual consenting or billing compliance … and have those across-the-aisle conversations, understanding can take leaps forward," Schick continued. "I think that helps normalize and bring certain change efforts into the mainstream."

MAGI has also furthered conversations on optimizing the study startup process, a discussion that's been going on since at least 2009, including the use of smart forms to make startup efforts easier, says Jennifer Peterson, director and head of clinical quality at M3 Wake Research, a 26-site network in the U.S.

More recently, MAGI has helped make strides in industry's move from paper to eRegulatory in clinical trials. Sites, sponsors and CROs have taken incremental steps when it comes to the adoption and use of eRegulatory systems, and this space has seen its share of challenges, she said. MAGI has served as a platform to openly discuss these struggles and potential solutions.

"One of our sessions within MAGI@home was about the complexities of moving from paper to eRegulatory and having that type of openness of 'what are your issues, what are your challenges?' People … sharing those types of issues that they've been having are just an example of what happens even in sidebar conversations in the hallways at MAGI," she said. "It's all about the practical sharing of knowledge."

"Everyone wants to share their passion," Peterson added, "and that is what has made this conference exciting and what has always made me want to return."

Listen to the full podcast episode here.

Register for the next MAGI conference here.

## Data

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will lead to a proliferation in data types (including an increased focus on genomic data) and more adaptive trial designs. Data collection — and hopefully data-sharing — will take center stage next year, enabling us to pursue additional research questions and perform meta-analyses.

Despite all the hype around AI and large language models, the key for AI success is our ability to access volumes of well-harmonized, governed, real-world data. With more personalized approaches, clinical trials grow more complex, requiring, again, better modeling and data collection plus a reliance on modern day data engineering and data scientists to identify trends and understand the causality of the therapeutic interventions in question.

The side effects of the pandemic will continue to unfold in 2024 as well. We will see more remote monitoring of patients, as well as the adoption of more digital health technologies, including mobile apps and specialized devices. Collecting and gaining insights from these data sources, coupled with the continuing trend of decentralized clinical trials, will require data strategies from both sponsors and CROs that leverage cloud computing and data governance at a scale that is much different than today.

**Stephen Ohnmacht**
Vice President, R&D Business Consulting, Europe
Veeva Systems

The life sciences industry has been waiting a long time for big data to transform the commercial viability of personalized medicine. With automation now coming of age, research and development teams can finally seize the opportunity as long as their big data is clean, standardized, interoperable and secure.

In 2024, companies will focus on making big data (which could range from raw trial and site-specific data to IT data points, such as cycle times) more usable by resolving common pain points around cleaning, ownership and standards. As a result, the volume of study data will increase exponentially. This will require a transparent data model with stringent user access controls to address data privacy and cybersecurity concerns.

Leading companies will use automation to make hundreds of marginal and incremental efficiency gains across the development lifecycle, whether deep-querying protocols, detecting patterns during medical imaging analysis or verifying the origin of chemical components. A growing industry impetus will lead to more direct data interface among sponsors, health institutes and regulatory authorities so that "big (clean) data" becomes a reality, creating the right conditions for commercially viable, personalized medicines to reach patients in need.

**Jim Reilly**
Vice President, Development Cloud Strategy
Veeva Systems

As clinical research sites continue to consolidate and reduce staff in 2024, sponsors will have to compete for the highest-performing sites and principal investigators. They'll do this by building stronger relationships and streamlining their tech stacks to reduce site burden.

Since the pandemic, there's been an explosion of technology intended to give patients more technology options for clinical trials. While this is helpful for patients, it has translated into more technology for sites to deal with. It also placed more emphasis on where trials are being conducted rather than how trial data and information are collected and shared.

From these challenges, we expect to see sponsors investing more in site engagement strategies and site-facing technology and more investment in simplifying the end-to-end data flow. Not only will this approach streamline study execution and management, but it will also lessen the transactional nature of site-sponsor relationships.

**Richard Young**
Vice President, Vault CDMS Strategy
Veeva Systems

As the complexity of clinical research increases sharply in the coming years, we will realize new operating models for patients, sites and sponsors. To deliver new data and user journeys that connect all clinical research contributors, we will call time on disconnected tools and embrace the platform era.

In previous decades, the industry addressed challenges by throwing resources at every problem. When that didn't work, we created burdensome point solutions that lowered productivity. Sponsors undertaking today's complex trials, including in cell and gene therapy, will need data-driven connectivity so patients can participate effectively and trial data can be reviewed and actioned in real-time. This is only feasible when all relevant data can be managed on the same platform.

Data will be distributed across research participants in a controlled and appropriate manner. Instead of silos, each data point will automatically initiate the next step in clinical trial execution. Humans will remain in the loop even while there is less need for manual intervention and facilitation. This new level of connectivity will drive science forward by providing the flexibility to work with diverse, distributed and exponentially growing data sources.

## DCTs

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The consortium will conduct a granular analysis of actual data to gain a better understanding of the impact of specific clinical trial innovations to better inform future protocol design. With this information, the industry can take a more fit-for-purpose approach to determining how to incorporate decentralized elements into more trials. During COVID, we tried everything. Now, with more evidence, we can get better at matching decentralized innovations to populations, diseases and specific study designs. This could have the add-on effect of decreasing complexity — for instance, rather than trying to implement electronic clinical outcome assessments, wearables and home-health nurses in the same trial (which may be overkill), we can carefully plan to use just the technologies that are most likely to improve outcomes.

As we continue to capture evidence of DCT value, we will increasingly adopt a nuanced approach, but this progression will move at the pace of maturity of each DCT innovation. By the end of 2024, the life sciences industry won't distinguish DCTs from site-based trials anymore. As noted by the FDA, the modernization of clinical trials is an ongoing evolution. In the U.S., it will require an act of Congress to change the definition because the term DCT is used in provisions published by the Food and Drug Omnibus Reform Act of 2022 (FDORA. Other regulators are already talking about trials with decentralized elements (i.e., EMA's recommendations paper) rather than DCTs.

Even with existing regulatory nomenclature, the acronym DCT will become less of a call-out than a distinct type of unique clinical trial, and that is a step in the right direction. The term suggests that a DCT is uncommon and requires special extra adjustments, but that is not true. Recent FDA and other global regulatory agency guidances explicitly note that DCTs simply need to comply with existing regulations for clinical trials – nothing extraordinary. There may be some data quality/privacy/security considerations, but the existing playbook still applies to trials leveraging decentralized innovations.

By this time next year, DCTs will simply be clinical trials (with certain parts appropriately decentralized) and the impact of this model will be much more prevalent.

**Alison Holland**
Executive GM of Customer Value
Medable

Over the past 18 months, the industry has spent a lot of time trying to define DCTs and debating what a DCT consists of, but now we are focused on outcomes. DCT methodologies give us more options around evidence collection – opportunities for data capture in real time – all with greater integrity and less friction for the patient. Now as we ring in a new year, we are simply working to decide what elements should be decentralized to get the desired outcome. With more choices about how to package a clinical trial ecosystem, we can make research more of a consumer-like process.

For 2024 and beyond, there will be an increase in focus on patient diversity in clinical trials. The difficulties of a lack of patient representation in clinical research bubbled up to the surface and became a raging boil during the pandemic's vaccine trials, and that issue isn't going away. Some of the approaches may change from what we have learned – for instance, there is far more capacity now to connect with broader patient populations using new digital technologies. Given the convenience of these tools, more patients who become increasingly comfortable with technology will take advantage of digital opportunities to connect to a trial and remain engaged until the end. This will also help ensure patient compliance for the full duration of a trial even when patience and perseverance start to wane in the later weeks and make it harder to capture consistent data.

The pharmaceutical marketplace is more competitive than ever, particularly in the race to be first to market. For instance, the time between the first FDA approval for a vaccine for RSV to the fourth FDA approval for a similar vaccine was less than six weeks. The first-mover advantage is dramatic, so clinical trial sponsors are hyper-focused on making sure they are maximizing every second and not wasting time. To that end, large pharmaceutical companies are setting lofty goals of 50 percent reductions in cycle time next year; the status quo won't allow them to reach such significant new goals. Sponsors will need to make commensurate changes to accelerate drug development.

DCT methodologies will now be part of the standard toolkit, embedded into most trial processes and operational decisions because sponsors need new ways to engage with patients in a competitive marketplace and collect data reliably. In 2024, the shift to DCT methodologies will be transformative and wholesale, bringing DCT elements into the organization at the portfolio level rather than trial by trial.

Decentralized methodologies allow for higher-fidelity data collection and greater objectivity in signal detection, removing skews and biases. This enables a more informed understanding of an investigational medication's safety and efficacy, which allows companies to reduce the sample size, and fewer participants means less patient recruitment time and lower costs. Roche reduced its sample size by 70 percent this way, helping to speed drug development and time to market.

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## DCTs

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**Joel Morse**
CEO and Cofounder
Curavit Clinical Research

DTx companies will continue to pivot away from prescription models. In 2023, Pear Therapeutics filed for bankruptcy and Akili abandoned the prescription business model. Both DTx companies raised hundreds of millions of dollars by going public in Special Purpose Acquisition Company (SPAC) transactions. While each company successfully gained FDA approvals, the current market acceptance for prescription DTx productsis low and improvements will take years. Given this, many DTx companies will pivot away from the prescription model and go straight to consumers next year. They will continue to run clinical trials to confirm their claims and value proposition but will not seek FDA approvals.

Health economics and outcomes research (HEOR) will become more common in decentralized DCTs. This year, we saw the maturation of claims and health information exchanges, which enable a cost-effective and straightforward process for researchers to gain access to identified patients' data. Given the improvements in costs, trial sponsors can now take advantage of these exchanges at scale and leverage the data for both prescreening and health economic analysis. As DCTs and virtual site acceptance accelerates, a knock-on effect will be that more of these trials will include HEOR analysis.

## Guest Column

as young doctor stricken with a near fatal auto-inflammatory disease, as he describes in his book, "Chasing My Cure."

Now, to help many others, he has established Everycure, a non-profit drug repurposing effort that combines global collaboration, artificial intelligence, and machine learning to survey drugs already approved and match them to possible new indications.

His team is finding matches and running trials. So far, this work has resulted in the treatment of 13 people with newly repurposed medications. In addition to lives saved, Everycure's efforts have shown proof of concept for a massively important model for the future of drug development.

When Fajgenbaum presented recently, he read a quote from FDA Principal Deputy Commissioner Janet Woodcock: "Sadly, no one is responsible for making sure that drugs are fully utilized across diseases. Paradoxically, once a drug is approved … all hope is lost that it will be studied and used in all diseases it can treat."

Taking responsibility to do his part to change that, Fajgenbaum sees a scaling up as other researchers adopt these methods. "We are not only working on the saving of lives today but showing a way forward to significantly increase the rate of new drug approvals.

It could be that the days of searching through medical journals, article by article, could be left behind, as AI and machine learning rise up. Fajgenbaum's success is a call to action to other researchers and drug companies to embrace drug repurposing.

# 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** | | |
| Asklepios BioPharmaceutical | AB-1005 | Multiple system atrophy-parkinsonian type |
| Chengdu Origen Biotechnology<br><br>Vanotech | KH631 | Wet age-related macular degeneration |
| Inipharm | INI-822 | Fibrotic liver diseases |
| Longboard Pharmaceuticals | LP659 | Rare neuroinflammatory conditions |
| Neumora Therapeutics | NMRA-266 | Schizophrenia/neuropsychiatric disorders |
| OncoNano Medicine | ONM-501 | Advanced solid tumors and lymphomas |
| Orionis Biosciences | ORB-011 | Advanced solid tumors |
| Plexium | PLX-4545 | Tumors refractory to checkpoint inhibitors |
| SpyBiotech | SPYVLP01 | Human cytomegalovirus vaccine |
| TOLREMO therapeutics | TT125-802 | Solid tumors |
| **phase 1a** | | |
| ProMIS Neurosciences | PMN310 | Alzheimer's disease |
| **phase 1/1b** | | |
| Dragonfly Therapeutics | DF6215 | Advanced solid tumors |
| **phase 1b** | | |
| Incendia Therapeutics | PRTH-101 plus pembrolizumab | Advanced or metastatic solid tumors |
| **phase 1b/2a** | | |
| Benitec Biopharma | BB-301 gene therapy | Oculopharyngeal muscular dystrophy-related dysphagia |
| Jasper Therapeutics | Briquilimab | Chronic spontaneous urticaria |
| **phase 1/2a** | | |
| ASC Therapeutics | ASC618 gene therapy | Moderate-to-severe hemophilia A |
| BioInvent | BI-1910 alone and with pembrolizumab | Solid tumors |
| Ceapro | Avenanthramides | Inflammation-based diseases |
| **phase 1/2** | | |
| Alentis Therapeutics | ALE.C04 | CLDN1+ tumors |

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## Study Lead Opportunities continued from page 7

| Company name | Drug name | Indication |
|---|---|---|
| **phase 1/2** | | |
| Ceapro | Avenanthramides | Inflammatory diseases |
| Hemab Therapeutics | HMB-001 | Glanzmann thrombasthenia |
| Neurogene | NGN-401 | Rett syndrome in female pediatric patients |
| Ocugen | OCU410ST gene therapy | Stargardt disease |
| Ocugen | OCU410 gene therapy | Geographic atrophy secondary to dry age-related macular degeneration |
| OncoResponse | OR502 alone and with anti-PD-1 | Advanced solid tumors |
| Vaxcyte | VAX-31 31-valent pneumococcal conjugate vaccine | Invasive pneumococcal disease in adults |
| **phase 2** | | |
| Acadia Pharmaceuticals | ACP-204 | Hallucinations and delusions associated with Alzheimer's disease psychosis |
| Bolt Biotherapeutics | BDC-1001 | HER2+ breast cancer patients who progress after Enhertu |
| Carmot Therapeutics | CT-868 | Type 1 diabetes in overweight or obese adults |
| Cerevance | CVN424 | Early-stage Parkinson's disease |
| CG Pharmaceuticals | Ivaltinostat | Metastatic pancreatic cancer |
| Glycomine | GLM101 | Phosphomannomutase 2-congenital disorder of glycosylation in pediatric patients |
| Kymera Therapeutics<br><br>Sanofi | KT-474 | Moderate-to-severe atopic dermatitis |
| MC2 Therapeutics | MC2-25 VLS | Vulvar lichen sclerosis |
| Skye Bioscience | SBI-100 Ophthalmic Emulsion | Primary open-angle glaucoma or ocular hypertension |
| Trishula Therapeutics | TTX-030 | Metastatic pancreatic ductal adenocarcinoma |
| UNITY Biotechnology | UBX1325 | Diabetic macular edema |
| Vergent Bioscience | VGT-309 | Imaging agent in lung cancer surgeries |
| **phase 2a** | | |
| AltruBio | ALTB-268 | Ulcerative colitis |
| Cocrystal Pharma | CC-42344 | Pandemic and seasonal influenza A |
| Escient Pharmaceuticals | EP262 | Atopic dermatitis |
| GRI Bio | GRI-0621 | Idiopathic pulmonary fibrosis |
| Halia Therapeutics | HT-6184 | Lower-risk myelodysplastic syndromes |
| Opsidio | OpSCF | Atopic dermatitis |

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## Study Lead Opportunities continued from page 8

| Company name | Drug name | Indication |
|---|---|---|
| **phase 2b** | | |
| Lexicon Pharmaceuticals | LX9211 | Diabetic peripheral neuropathic pain |
| Trevi Therapeutics | Haduvio (oral nalbuphine ER) | Chronic cough in idiopathic pulmonary fibrosis patients |
| **phase 2/3** | | |
| Exelixis | Zanzalintinib plus pembrolizumab | Previously untreated recurrent or metastatic head and neck cancer |
| **phase 3** | | |
| Acadia Pharmaceuticals | ACP-101 (carbetocin nasal spray) | Hyperphagia in Prader-Willi syndrome |
| Aquestive Therapeutics | Anaphylm (epinephrine) sublingual film | Severe life-threatening allergic reactions |
| Aura Biosciences | Bel-sar (belzupacap sarotalocan) | Early-stage choroidal melanoma |
| Bayer | Asundexian (BAY2433334) | Patients aged ≥65 years with atrial fibrillation ineligible for oral anticoagulant treatment |
| BridgeBio | Infigratinib | Achondroplasia in pediatric patients |
| Clarity Pharmaceutical | 64Cu-SAR-bisPSMA | Detection of regional nodal metastasis in high-risk prostate cancer patients prior to radical prostatectomy |
| DARÉ Bioscience | Ovaprene | Contraceptive |
| JCR Pharmaceuticals | JR 441 | Mucopolysaccharidosis type IIIA |
| Polaris Group | ADI-PEG 20 | Leiomyosarcoma |
| Sol-Gel Technologies | SGT-610 (patidegib gel, 2%) | Gorlin syndrome |
| Telix Pharmaceuticals | TLX591 (177Lu-rosopatamab tetraxetan) | PSMA-positive metastatic castrate-resistant prostate cancer |
| Verastem Oncology | Avutometinib and defactinib | Recurrent low-grade serous ovarian cancer |

# 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 |
|---|---|---|---|
| Alzamend Neuro | AL001 | Major depressive disorder | IND approved |
| Alzamend Neuro | AL001 | Post-traumatic stress disorder | IND approved |
| Carisma Therapeutics | CT-0525 | HER2+ solid tumors | IND approved |
| CARsgen Therapeutics | CT071 | Relapsed/refractory multiple myeloma and relapsed/refractory primary plasma cell leukemia | IND approved |
| Century Therapeutics | CNTY-101 | Systemic lupus erythematosus | IND approved |
| Defence Therapeutics | ACCUM-002 Dimer CDCA-SV40 with and without nivolumab/ relatlimab | Solid tumors | IND approved |
| GigaGen | GIGA-564 | Solid tumors | IND approved |
| Gracell Biotechnologies | FasTCAR-T GC012F | Refractory systemic lupus erythematosus | IND approved |
| GRIbio | GRI-0621 | Idiopathic pulmonary fibrosis | IND approved |
| HOOKIPA Pharma | HB-500 | HIV | IND approved |
| Immuneering | IMM-6-415 | Advanced RAF or RAS mutant solid tumors | IND approved |
| Inflammasome Therapeutics | Kamuvudines | Geographic atrophy | IND approved |
| KSQ Therapeutics | KSQ-001EX | Melanoma, head and neck squamous cell carcinoma and non-small cell lung cancer | IND approved |
| Lighthouse Pharmaceuticals | LHP588 | P. gingivalis-positive Alzheimer's disease | IND approved |
| MyMD Pharmaceuticals | MYMD-1 | Rheumatoid arthritis | IND approved |
| Nexcella | BCMA CAR-T NXC-201 | Relapsed/refractory AL amyloidosis | IND approved |
| Omnix Medical | OMN6 | Hospital-acquired bacterial pneumonia or ventilator-associated bacterial pneumonia | IND approved |
| Senhwa Biosciences | CX-4945 | Community-acquired pneumonia caused by viral infection | IND approved |
| Soligenix | SGX945 | Aphthous ulcers in Behçet's disease | IND approved |
| TC BioPharm | TCB008 | Relapsed/refractory acute myeloid leukemia | IND approved |
| Tenax Therapeutics | TNX-103 (oral levosimendan) | Pulmonary hypertension with heart failure with preserved ejection fraction | IND approved |
| Vittoria Biotherapeutics | VIPER-101 | Relapsed/refractory T-cell lymphoma | IND approved |

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## FDA Actions continued from page 10

| Company name | Drug name | Indication | FDA action |
|---|---|---|---|
| RTI Surgical | Cortiva Allograft Dermis | Breast reconstruction | IDE approved |
| AstraZeneca | Truqap (capivasertib) plus Faslodex (fulvestrant) | HR+ HER2- locally advanced or metastatic breast cancer with PIK3CA, AKT1 or PTEN alterations | Approved |
| bluebird bio | Lyfgenia (lovo-cel) | Sickle cell disease | Approved |
| Bristol Myers Squibb | Augtyro (repotrectinib) | Locally advanced or metastatic ROS1-positive non-small cell lung cancer | Approved |
| Novartis | Fabhalta (iptacopan) | Paroxysmal nocturnal hemoglobinuria | Approved |
| SpringWorks Therapeutics | Ogsiveo (nirogacestat) | Desmoid tumors | Approved |
| Takeda Pharmaceuticals | Adzynma (ADAMTS13, recombinant-krhn) | Congenital thrombotic thrombocytopenic purpura | Approved |
| Valneva Austria | Ixchiq (chikungunya vaccine) | Chikungunya virus | Approved |
| Vertex Pharmaceuticals<br><br>CRISPR Therapeutics | Casgevy (exagamglogene autotemcel) | Sickle cell disease | Approved |
| Astellas Pharma | Cresemba (isavuconazole) | Invasive aspergillosis and invasive mucormycosis | Approved in new formulation and for expanded age indication |
| Astellas<br><br>Pfizer | XTANDI (enzalutamide) | Nonmetastatic castration-sensitive prostate cancer with biochemical recurrence at high risk for metastasis | Approved for expanded indication |
| Merck | Keytruda (pembrolizumab) | First-line locally advanced unresectable or metastatic HER2-negative gastric or gastroesophageal junction adenocarcinoma | Approved for expanded indication |
| Eli Lilly | Jaypirca (pirtobrutinib) | Chronic lymphocytic leukemia or small lymphocytic lymphoma patients who have received at least two prior lines of therapy, including BTK and BCL-2 inhibitors | Approved for a new indication |
| CorMedix | DefenCath (taurolidine and heparin) catheter lock solution | Catheter-related bloodstream infections | Approved |
| Medtronic | Symplicity Spyral renal denervation system | Hypertension | Approved |

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