The CenterWatch Monthly

In This Issue

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Mobile Trial Vans, Research Paramedics and More Featured in WCG Innovation Challenge

By James Miessler

The 2024 MAGI Clinical Research Conference in New Orleans played host to yet another exciting round of clinical trial innovation, with attendees voting on their favorite of five creative approaches in the WCG Innovation Challenge. Read on to learn more about this year's winner as well as the four finalists.

Winner — Research Works

Research Works, an integrated research organization headquartered in New Orleans, was crowned the winner of the WCG Innovation Challenge for its use of a converted Mercedes Sprinter van as a clinical trial on wheels.

Three years after taking on irritable bowel syndrome trials in 2017 and encountering serious challenges in bringing participants to the site, Research Works began considering decentralization and virtual home visits as potential solutions.

Kaye Doiron, founder and CEO, came up with a bold and innovative idea that year: purchase a Mercedes Sprinter van and turn it into a comprehensive trial vehicle that serves "basically [as] an annex of the research site," she told *The CenterWatch Monthly*.

"The 'hub and spoke' model for us is a failing model alone. It's exponentially costly to build and a beast of a burden to manage," Doiron told conference attendees. "[Our van] brings the clinical trial site directly to the patient at home as a standup pop-up site or seamlessly integrated into medical practice."

"It depends on the target population you're going after and how creative you want to be with your marketing scheme," Doiron said. "We recently ran an STD study and it was a huge impact to be able to go park on Sorority Row or a college campus to get young people to come and read about our study."

The van serves as a full pharmacy and laboratory on wheels, equipped with everything Research Works needs to conduct trials: a refrigerator and medical freezers; an ambient cabinet; an intravenous infusion chair and accompanying cardiac equipment; rescue equipment; and an EKG machine. It's also equipped with curtains for blinding and unblinding patients.

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Align eSource Choices With Site Needs, Expert Advises

By James Miessler

Making the move to eSource can be a daunting task for a site, making it important to understand what each potential choice offers and how they align with your site's needs, one trial operations expert advises.

An eSource system could be designed to serve as a storage space for paper source documents that are uploaded and stored electronically, says Ellen Neylon, clinical operations manager at Palm Research Center, a Las Vegas-based research institute, or a direct data entry system that automatically transfers into an EDC system, or a combination of the two.

Consider the trials and therapeutic areas your site covers and what their specific data requirements are when evaluating eSource platforms, Neylon advised attendees at the MAGI Clinical Research Conference in New Orleans last week.

Sites with endocrinology trials, for example, will have many things to consider when choosing an eSource system, such as EKGs that print out on paper, a mix of electronic and paper patient-reported outcomes, and a combination of eDiaries and paper diaries. Diabetes trials, on the other hand, involve lengthy wearable device downloads that can reach up to 50 pages, in Palm Research Center's case.

"How is [all] that transferred into eSource? Everyone has a different answer

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Going From Strain to Success with Site Coverage Analyses

Whether sites have the resources to devote to coverage analysis, these evaluations lay the groundwork for trial budgets. ***Sandy Smith****, WCG senior vice president of clinical solutions and strategic partnering, tackles how to properly conduct and manage coverage analysis amidst site staffing constraints.*

With recent headlines drawing attention to billing compliance errors and their consequences, sites are focusing even more on developing detailed workflows for creating research budgets and ensuring compliant processes for precise sponsor billing and payer billing.

Compliant budget creation starts with completing a thorough review of the trial protocol and determining routine care vs. research-specific elements — this is called a coverage analysis. It is sometimes referred to as a Medicare coverage analysis, as Medicare reimbursement guidelines (National and Local Coverage Determinations) are used as the benchmark, but other resources, such as National Comprehensive Cancer Network (NCCN) guidelines, may also be used to support decisions on routine vs. research elements.

The Challenge: The tumult in research staffing has impacted sites, leaving them understaffed or potentially without the skill set to make coverage determinations. As the coverage analysis is the foundation for creating the trial budget, it is imperative that it be done thoroughly and promptly. If experienced staff are not available to perform this vital function, the question becomes whether trial activation halts or the site continues forward, running the risk of noncompliant billing.

The Solutions: There are two options to consider: buy or build.

"Buy" would involve purchasing services from a third party on an interim basis or for the long-term. This allows the site to keep trial activation moving forward. When evaluating a third-party partner, there are several questions to consider:

- **Reputation:** How long has the third party provided this service? Is it willing to provide references from other sites purchasing these same services?
- **Team:** Are the people providing the service employees of the third party or are they contractors? Are they U.S.-based, allowing for direct contact with research site team members during normal working hours?
- **Cost:** Is the fee structure well-defined? Is it a flat fee or hourly (variable)?
- **Process:** How does the site get started? Is there an onboarding process? How does communication flow while the work is being performed? How does the third party understand and incorporate site preferences? When amendments are issued for the protocol, how are these managed? How long does it take for the third party to complete the work product? How does the site communicate changes to be made? How is investigator input considered?

Reputable third-party partners should answer all of these questions, allowing the site to modify workflows as needed, plan for expenses and anticipate turnaround times in their startup processes.

The "build" option can be lengthy – the time to post, recruit, interview, hire and onboard a new team member could take months – but it can be accomplished.

Consider how you would onboard a new hire. Who would conduct the training? Does anyone at the site have the time and expertise to train and oversee the orientation of a new team member? If it is a small site, there may only be one person or position for this function. There are online resources and workshops to learn these skills, but it takes time to build this skillset. Networking with people at other sites performing this function could also be considered.

The potential impact of omitting a coverage analysis for trials in which routine care is part of the protocol could have severe implications, from noncompliant billing to not being fully reimbursed for all care provided as part of the clinical trial. This important function should never be overlooked!

Novice Researchers and Sites Deserve Equal Consideration: The Story of a Standout Performance

Elizabeth Weeks-Rowe, *veteran CRA, trainer and published author, shares her experience working with a new but earnest principal investigator whose stellar site evaluation visit and diligent preparation earned her praise and recommendation.*

Investigator and site evaluation is a critical CRA responsibility that requires finesse to fully understand the diversity of investigational site models and personnel for appropriate study selection. As there is no standard one-size-fits-all site, there is likewise no standard site template to uniformly determine capabilities across the site landscape.

Each investigator and site is unique and merits a holistic evaluation process to determine if they fulfill the FDA requirement to select "trained and qualified investigators" for study conduct. This includes both experienced and new investigators and sites; each have equally valuable attributes to contribute to study conduct.

Though shortsighted, new investigators and sites are sometimes automatically disregarded due to lack of clinical research experience. A new investigator may lack the required clinical trial experience but may alternatively possess advanced clinical or scientific training to supplement the lack of formal research experience. Common sense, creativity and raw instinct developed during clinical training may serve as a critical foundation for assimilating clinical research regulations and guidances for the role.

It is critical that new sites be given the same level of consideration for trial participation. The evaluation visit will require an additional line of questioning to fully understand the research-naïve site capabilities and supplemental attributes but is well worth the additional investment to discover and appropriately develop a potential site partner for their program.

I have conducted hundreds of site evaluation visits over the course of my career, and one of the most impressive evaluations occurred with a new investigator who had gone to extraordinary lengths to prepare his site for the visit. As a result, site staff demonstrated enthusiasm and due diligence in every aspect of readiness. Their efforts fully aligned with the protocol, FDA regulations and industry best practices.

I arrived at the research site and was immediately impressed with the presentation of personnel and equipment. Though the space was modest and the equipment older, everything was clean and maintained perfectly.

The investigator had enlisted the assistance of an experienced PI, a physician colleague, to serve as his mentor and guide as he set up his site infrastructure and learned his new role. The investigator had created standard operating procedures (SOP) for critical research processes consistent with GCP principles, including informed consent conduct, investigational product (IP) receipt, storage and administration, staff training, study patient screening/enrollment, etc.

He had hired an experienced study coordinator and enlisted one of his physician assistants to serve as a sub-investigator. The PI advising them had recommended a training plan that covered GCP and human subject protection, IRB and investigator responsibilities, informed consent, protocol review and serious adverse event/safety reporting, as well as review of ICH E6 guidelines and FDA. The advising PI had also committed to guiding site staff through screening of the first three patients for the study.

The investigator had acquired and/or refurbished equipment for standard study procedures, including a centrifuge; a -20C freezer; calibrated vital signs monitoring equipment; a locked cabinet for IP storage with a web-based, continuous temperature monitoring system; an EKG machine; a blood glucose monitor; and a scale. The investigator had researched the protocol procedures and schedule of assessments closely to ensure he had access to specialty diagnostic centers to support study endpoints.

Beyond attending the protocol discussion, as is customary practice, the investigator personally hosted the entire three-hour evaluation visit. The study coordinator and sub-investigator were also in attendance and provided valuable insight to the site's patient population and enrollment practices. They had organized all site training certificates and equipment calibration records in a three-ring binder that they presented to me at the end of the visit.

The staff started and ended the evaluation visit with grace at transparency; at the start of the visit the investigator had informed me he was new to clinical research and realized it was a large learning curve. At the end of the visit, he assured me his staff would continue their research training and familiarization to ensure patient safety and credible data. He thanked me graciously for my time and expressed his earnestness in being selected for our study.

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How Latinas Are Helping Shape Clinical Research

Latin American women are playing an important role in shaping clinical research. This article, excerpted from a blog post by WCG site services and Latin America specialists, takes a closer look at their obstacles and triumphs and gives voice to some successful Latina researchers.

Latinas engaged in clinical research encounter systemic biases and obstacles, including underrepresentation and restricted access to education, resources and opportunities. Data from the United Nations Educational, Scientific and Cultural Organization (UNESCO) and UN Women reveal that while women make up 45 percent of researchers in Latin America and the Caribbean, their representation in scientific fields remains at less than 30 percent.

Despite often filling roles that are less visible, many Latinas are making significant contributions to clinical research and serving as advocates for diversity, equity and inclusion. Their efforts typically take one (or more) of three forms.

Trailblazing Role Models and Mentors

Latinas in clinical research encounter systemic biases and obstacles, including underrepresentation and restricted access to education, resources and opportunities. Despite these challenges, many Latinas actively contribute to clinical research, often in less visible roles, serving as positive examples for future researchers.

Flor Caffarena, a WCG CRC, shares her guide and inspiration in clinical studies: her mother, Helena Maria Gandur. "Even before I started working in this field, I saw what she did and listened to her talk about clinical research; her work always caught my attention," she says. "I started working with her, sharing the same office, learning everything from her, and I consider her to be the best in her position, not only because of her performance, but because of the passion and love she puts into everything she does."

Claudia Uña, a former WCG CRC, was inspired her in her career path in the world of medicine by Argentinian scientist Cecilia Grierson, the first woman to graduate and receive a medical degree in 1859 in that country, overcoming the barriers in an originally male-dominated field. She was a pioneer in the promotion of public health and medical education in Argentina, improving medical access, especially with the most vulnerable populations. Her emphasis on professional training laid the first foundations for clinical research development in Argentina.

Community and Organizational Leadership

Latinas spearhead advocacy organizations and initiatives dedicated to advancing diversity and inclusion within clinical research. Through their leadership and activism, they drive meaningful change and create spaces for underrepresented voices to be heard.

Marinez Pugsley, a former WCG CRC, moderates CRC networking groups across multiple platforms and actively promotes professional networking both regionally and internationally. Dedicated to advancing the clinical research community, she ensures her network stays informed about training opportunities and job vacancies in the region. Pugsley says she has been inspired by the selfless support of clinical researcher Daniele Argentina, who helped refine her LinkedIn profile and continues to offer professional advice and insights.

Pugsley, along with Heda Amarante, a former WCG PI, annually organize a clinical research conference in Curitiba, Brazil, where they encourage open communication and knowledge-sharing on various trending research topics.

Using Personal Experiences

Latinas actively engage in research addressing health disparities and inequities within Latino communities. They champion inclusive and culturally competent approaches, ensuring that research endeavors accurately reflect and address the diverse needs of all communities.

"I enjoy the intellectual challenge and interdisciplinary collaboration that characterizes clinical research, as well as the sense of purpose that comes from contributing to the advancement of medical and scientific knowledge," says Alejandra Vidal, a former WCG CRC. "However, I think the most exciting thing is that, for example, today we may be working on the study of a therapy that, in the coming years, may mean the cure of a disease. And that is the great value of our work."

Overall, Latinas actively advocate for equity, diversity and inclusion within the field of clinical research, drawing inspiration from their personal experiences and the accomplishments of others. They pave the way for future generations of Latina researchers by championing these values.

In the words of Amarante: "I think that female health professionals, who are already in the majority in many countries, will be very prevalent in clinical research due to the compatibility of this work with the feminine characteristics of dedication, determination, professional ethics, correctness, appreciation for detail and quality of care with the patient, among others."

Read the full blog post here.

Innovation Challenge

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Its mobility gives it prime access to community happenings, such as church gatherings, festivals, concerts and university events, among many others. When not on the road, it plugs into Research Works' home base to serve as an extra room. The hybrid-engine vehicle runs up to 40 hours idling, 12 to 14 hours on battery backup and nonstop when plugged into a standard wall outlet.

The van also solves a state-specific problem — a growing number of physicians being barred contractually from running trials for outside research companies within their practices, according to Doiron. The van being its own separate unit makes it easy for physicians to conduct trials outside of their buildings without fear of violating their contracts.

Doiron's innovation didn't come together overnight. Designing the mobile trial van was a challenging endeavor, particularly when it came to the tighter confines of the van and the power needed to run all the trial equipment. Months of working with an engineering team were necessary to make her vision a sufficiently powered reality. Thankfully, the head of the team was an EMT accustomed to administering care in small spaces, and chair-related challenges were mostly solved through the use of a tattoo chair that can be laid flat for chest compressions, though Doiron intends to obtain a smaller chair in the future.

"Space was a big issue to really design a space where I can do an IV, manage cardiac responses, push rescue meds, get my staff around the chair to do chest compressions and defib. There was a lot of work that went into designing that space," Doiron pointed out. "The other [issue] was the power — getting the hybrid engine to be able to run a 70-below refrigerator, a 20-below refrigerator and

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Finalists

Four other finalists made it into the WCG Innovation Challenge and presented in New Orleans:

Wake Forest University School of Medicine, which countered staffing challenges with the use of research paramedics. Lauren Koehler, research manager, and Kylee Smith, research coordinator, explained how research paramedics possess skillsets well-suited to clinical research, including trust-building with patients and strong communication skills that enable them to talk to anyone. Koehler advises sites interested in this staffing model to check state requirements, as practice plans in some states don't allow the use of paramedics, and work with EMS leadership and paramedic programs to begin a conversation.

Erie Retina Research, whose innovative community clinic model not only boosts diversity and minimizes pretrial screening failures but also strengthens and invests in the surrounding community through thoughtful partnerships. Erie's director of clinical research, David Almeida, detailed how the site's work with food banks, schools, nonprofits and others to provide access to trials and standard-of-care assessments is centered on leaving the community better than when they arrived. The program has led to significant growth in screenings, enrollments and trial workload since its inception in 2021. This growth has been maintained through the use of an in-house risk management team comprised of a former FDA auditor, a chief monitor and a chief compliance officer that conducts random trial quality assessments on a monthly basis.

Cedar Health Research, a site network that uses innovative diversity strategies to integrate with community physician practices and ease the burdens of getting into clinical research. CEO Todd Albin told attendees how Cedar deploys an AI/machine learning solution that integrates with electronic health records to match patients to trials, helping sponsors significantly increase the diversity of their trial populations. The company pairs this with a healthcare partnership team that works one-on-one with physician practices to educate and inform them on clinical trials, as well as external marketing campaigns and community collaborations.

Louisiana State University Health New Orleans, whose virtual oncology platform has successfully extended trial access, improved adherence to trial procedures and eased travel burdens for female patients in rural areas of the state. Amelia Jernigan, division director of gynecology oncology at LSU Health New Orleans School of Medicine, shared how she and her team used a multidiscipline, tablet-powered survivorship care program already in place to launch a new program that connects travel-averse patients to trials. Through this effort, they have successfully enrolled patients who normally would have declined surgical oncology trials with follow up procedures that can be done locally.

Innovation Challenge

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an ambient cabinet. We don't ever turn this unit off."

It wasn't cheap, either, with the bill coming out to approximately three brick-and-mortar satellite sites with lab and pharmacy capabilities — nearly $195,000. But the payoff has been massive, Doiron says, and she urges those interested in crafting their own trial van not to be deterred by the initial costs. A comparator study conducted with three satellite sites, for example, showed that Research Works' mobile van boosted their patient database by 900 percent, and use of the van in a high-volume vaccine trial saw more than double the number of total consents compared to the traditional site.

"It is reliable, profitable, replicable and fully customizable for your sponsors' needs," she said. "As just an owner/founder with no outside capital, it took me four years to actualize it and realize it, but I was so motivated to get it built because I do feel it has limitless potential in many, many applications, not just conducting clinical trial research visits."

> "It is reliable, profitable, replicable and fully customizable for your sponsors' needs."
>
> — Kaye Doiron, Founder and CEO, Research Works

Doiron, who has plans to expand with more customized units, says she did face some pushback from skeptical sponsors, and she asks that sponsors have a more open mind when it comes to innovative approaches. Regulatory agencies, on the other hand, have been most excited about the van.

"Sponsors can't keep up with innovations fast enough to change their standard operating procedures or even think outside the box," she told *The CenterWatch Monthly*. "They need to be quicker to pivot for innovations that are coming in our industry."

To listen to recordings of MAGI 2024 sessions, click here.

eSource Adoption

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when I have spoken to different platforms and companies," Neylon said. "These are questions, as you're shopping for eSource, that you need to dig down into your workflow and figure out."

When Is It Time for eSource?

Many sites still haven't made the move to eSource, continuing to comfortably rely on paper methods. Could it be time for your site to make the transition? A number of things come into consideration when thinking about whether to adopt an eSource system, says Neylon. Consider:

- Is there a lack of standardized operations at the site(s), such as different sources at every site? Can eSource standardize site data capture?
- Are there an excessive number of systems in play at the site?
- Are there numerous users who require access to data, such as sponsor, CRO and site staff?
- Is the site satisfied with its current paper-based system?
- Will the use of eSource become mandatory at the site?
- Are there are any eSource platforms still in development that will be a better fit for the site once they're released?
- Is there a single platform that will provide everything the site needs?

Challenges and Considerations When Implementing eSource

There are significant burdens that come with implementing eSource. Determining the system that fits best is a lot of work, both functionally and financially, for example. The financial burden of investing in an eSource system may hit smaller sites much harder than larger sites, as buy-in for these small organizations can be much heavier. In addition, policies, standard operating procedures and site workflows will all need to be modified to line up with the new system. Experiences differ, too, when contracting with eSource providers and going through their help desks, and sites may need to dedicate a team to communicating with the eSource vendor.

In addition, sites will have to adhere to timelines for the new system and roll out training for all staff, principals and sub-investigators, as well as determine which trials to initially move, or not move, to the new technology. More financial and time demands will come, too, in the form of devices needed for on-site use — such as laptops, tablets and computers — and assessing the eSource platform's performance.

Technical challenges will also likely arise at some point as a site grows or downsizes. Suppose there's a good possibility your organization will add more sites in the future — is the system scalable?

The complexity of the platform matters, too. Neylon advises sites to consider if the system will require an IT team if something goes wrong and if your site even has an IT team to tend to issues. Similarly, every eSource platform will experience downtime at some point; think about how

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eSource Adoption

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that and Internet outages would impact patient care and what backup plan would be needed.

Data security is also a major concern with multiple facets, Neylon says:

- Security clearance for site staff: does the system limit the number of signed in employees? Does it limit where employees can sign in from? Some systems will only allow in-office log ins, for example.
- Infrastructure limitations:
  - Who at the vendor is confirming the product is 21 CFR Part 11 compliant?
  - Can the software handle the amount of data being gathered and uploaded?
  - Is there a limit on the amount of data storage? Is there a limit on how long data can be stored?
  - Who owns the data? How will the data be transferred when the contract with the eSource provider runs out?
- User privileges: How do you determine who among your staff will be given super user privileges? Who will be responsible for the system at the site?
- Monitor access: Is the system user-friendly enough to grant multiple monitors access to a trial during monitor visits?
- How is revoking access handled, both for site staff and monitors?

Moving to an eSource system can be very beneficial, but it also puts a lot at stake, especially for small sites, Neylon cautions, including data quality, sponsor contracts, trial performance, staff satisfaction and site reputation, and integration is hard to reverse once it's finalized. Overall, it's important to deeply consider: does the site really need it?

"[The] lift of the integration of this technology really falls on [a site's] current staffing," she said. "We really have to ask: if it's not broken, does it need to be fixed until a streamlined system [exists]?"

Viewpoint

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The investigator had gone above and beyond to reassure me that, despite his lack of research experience, he and his staff were dedicated to ethical study conduct and would continue their training to ensure they fulfilled this important obligation.

I recommended the investigator and his site for the study without hesitation.

Weeks-Rowe's most recent CenterWatch training guide, The PI's Guide to Conducting Clinical Research (3rd Edition), *is available here.*

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		
23andMe	23ME-01473	Advanced solid tumors
Aeovian Pharmaceuticals	AV078	Seizures associated with tuberous sclerosis complex
CinFina Pharma	CIN-110	Obesity
Corbus Pharmaceuticals	CRB-701	Advanced solid tumors associated with high Nectin-4 expression
Corvus Pharmaceuticals	Soquelitinib	Moderate-to-severe atopic dermatitis
CytomX Therapeutics	CX-2051	Advanced solid tumors
Iambic Therapeutics	IAM1363	HER2-driven cancers
Lantern Pharmaceuticals	LP-284	Relapsed/refractory non-Hodgkin's lymphoma and other solid tumors
MapLight Therapeutics	ML-007 plus a precision-matched peripherally active anticholinergic	Schizophrenia and Alzheimer's disease psychosis
Neurocrine Biosciences	NBI-1065890	Neurological and neuropsychiatric conditions
Public Health Vaccines	PHV01 Marburg virus vaccine	Immunization against Marburg virus
Rhythm Pharmaceuticals	RM-718	Obesity and hypothalamic obesity
Vanqua Bio	VQ-101	Parkinson's disease with GBA1 mutations
Valneva	VLA1601 Zika virus vaccine	Immunization against Zika virus
phase 1b		
Tectonic Therapeutic	TX45	Group 2 pulmonary hypertension in patients secondary to heart failure with preserved ejection fraction
ViGeneron	VG901 gene therapy	Retinitis pigmentosa caused by mutations in the CNGA1 gene
phase 1b/2		
Jasper Therapeutics	Subcutaneous briquilimab	Chronic inducible urticaria
phase 1/2		
Nuvation Bio	NUV-1511	Advanced solid tumors
phase 1/2a		
Immuneering	IMM-6-415	Advanced solid tumors with RAF or RAS mutations
phase 2		
Alto Neuroscience	ALTO-203	Major depressive disorder
Anavex Life Sciences	ANAVEX 3-71	Schizophrenia

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Study Lead Opportunities continued from page 8

Company name	Drug name	Indication
phase 2 continued		
Biophytis	BIO101 plus GLP-1 receptor agonists	Muscle loss due to obesity treatment with GLP-1 receptor agonists
Cybin	CYB004	Generalized anxiety disorder
Eureka Therapeutics	ARTEMIS ECT204 T-cell therapy	GPC3-positive advanced hepatocellular carcinoma
IntelGenx	Montelukast VersaFilm	Parkinson's disease
Molecure	OATD-01	Active pulmonary sarcoidosis
Neurocrine Biosciences	NBI-1070770	Major depressive disorder
PMV Pharmaceuticals	Rezatapopt	Advanced solid tumors harboring a TP53 Y220C mutation and KRAS wild-type
ProLynx	PLX038	Locally advanced or metastatic triple-negative breast cancer
Qlaris Bio	QLS 111	Primary open-angle glaucoma and/or ocular hypertension
Qlaris Bio	QLS 111 plus latanoprost	Open-angle glaucoma and/or ocular hypertension in patients aged 12 and up
Shaperon	NuGel	Atopic dermatitis
phase 2a		
Aligos Therapeutics	ALG-055009	Metabolic dysfunction-associated steatohepatitis
BerGenBio	BGBC016	First-line non-small cell lung cancer with STK11 mutation
LyGenesis	LYG-LIV-001 allogeneic cell therapy	End-stage liver disease
phase 2/3		
Daiichi Sankyo Merck	Raludotatug deruxtecan	Platinum-resistant ovarian cancer
Vertex Pharmaceuticals	Inaxaplin	APOL1-mediated kidney disease
phase 3		
Axsome Therapeutics	Solriamfetol	Major depressive disorder
Axsome Therapeutics	Solriamfetol	Binge eating disorder in adults
Harmony Biosciences	Pitolisant	Excessive daytime sleepiness and behavioral symptoms in patients aged 6 and up with Prader-Willi syndrome
Innovent Biologics	IBI310	Resectable MSI-H/dMMR colon cancer stage cT4 or cN+ neoadjuvant therapy
Intellia Therapeutics	NTLA-2001	Transthyretin amyloidosis with cardiomyopathy
Merck	Keytruda plus MK-1084	First-line treatment of certain patients with PD-L1 expressing metastatic non-small cell lung cancer with KRAS G12C mutations
Vistagen Therapeutics	Fasedienol	Acute treatment of social anxiety disorder

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
BioCity Biopharma	BC2027	GPC3 expressing cancers	IND approved
Bio-Thera Solutions	BAT8006	Platinum-resistant epithelial ovarian, fallopian tube or primary peritoneal cancer	IND approved
Delta-Fly Pharma	DFP-10917 plus Venetoclax	Relapsed/refractory acute myeloid leukemia	IND approved
DepYmed	DPM-1003	Rett syndrome	IND approved
IASO Biotechnology	Equecabtagene autoleucel	Generalized myasthenia gravis	IND approved
NMD Pharma	NMD670	AChR or MuSK antibody positive generalized myasthenia gravis	IND approved
Ocugen	OCU400 gene therapy	Retinitis pigmentosa independent of genetic mutation	IND approved
Oryzon Genomics	Iadademstat plus immune checkpoint inhibitors	First-line extensive stage small cell lung cancer	IND approved
Renexxion Ireland Dr. Falk Pharma	Naronapride	Gastroparesis	IND approved
SpliSense	SPL84	Cystic fibrosis	IND approved
Fractyl Health	Revita	Durable weight maintenance after discontinuation of GLP-1 based drugs	IDE approved
Akebia Therapeutics	Vafseo (vadadustat)	Anemia due to chronic kidney disease in adults on dialysis	Approved
AstraZeneca	Voydeya (danicopan)	Add-on therapy to ravulizumab or eculizumab for the treatment of extravascular hemolysis in adults with paroxysmal nocturnal hemoglobinuria	Approved
Basilea Pharmaceuticals	Zevtera (ceftobiprole medocaril sodium for injection)	Staphylococcus aureus bloodstream infections, in adults with right-sided infective endocarditis and acute bacterial skin and skin structure infections and for patients 3 months to less than 18 years old with community-acquired bacterial pneumonia	Approved
BeiGene	Tevimbra (tislelizumab-jsgr)	Unresectable or metastatic esophageal squamous cell carcinoma	Approved
Idorsia Pharmaceuticals	Tryvio (aprocitentan)	Uncontrolled hypertension	Approved
Italfarmaco	Duvyzat (givinostat)	Patients aged 6 and older with Duchenne muscular dystrophy	Approved
Johnson & Johnson	Opsynvi (macitentan and tadalafil)	Pulmonary arterial hypertension	Approved
Madrigal Pharmaceuticals	Rezdiffra (resmetirom)	Metabolic dysfunction-associated steatohepatitis	Approved
Merck	Winrevair (sotatercept-csrk)	Pulmonary arterial hypertension	Approved

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Company name	Drug name	Indication	FDA action
Orchard Therapeutics	Lenmeldy (atidarsagene autotemcel)	Metachromatic leukodystrophy	Approved
Alexion	Ultomiris (ravulizumab-cwvz)	AQP4 antibody-positive neuromyelitis optica spectrum disorder	Approved for new indication
AstraZeneca Daiichi Sankyo	Enhertu (fam-trastuzumab deruxtecan-nxki)	Unresectable or metastatic HER2+ solid tumors	Approved for new indication
Bristol Myers Squibb	Breyanzi (lisocabtagene maraleucel)	Relapsed/refractory chronic lymphocytic leukemia or small lymphocytic lymphoma	Approved for new indications
Vanda Pharmaceuticals	Fanapt (iloperidone)	Acute treatment of manic or mixed episodes associated with bipolar I disorder in adults	Approved for new indication
2seventy bio Bristol Myers Squibb	Abecma (idecabtagene vicleucel)	Relapsed/refractory multiple myeloma after two or more prior lines of therapy	Approved for expanded indication
Johnson & Johnson Legend Biotech	Carvykti (ciltacabtagene autoleucel)	Relapsed/refractory multiple myeloma after at least one prior line of therapy	Approved for expanded indication
Medexus Pharmaceuticals	Ixinity [coagulation factor IX (recombinant)]	On-demand, prophylactic and perioperative treatment of patients under 12 with hemophilia B	Approved for expanded indication
ViiV Healthcare	Dovato (dolutegravir/ lamivudine)	HIV-1 infection in adolescents aged 12 and up weighing at least 55 lbs	Approved for expanded indication
Boehringer Ingelheim	Spevigo (spesolimab-sbzo)	Generalized pustular psoriasis in patients aged 12 and up weighing at least 88 lbs	Approved for expanded population
Esperion	Nexletol (bempedoic acid) tablets and Nexlizet (bempedoic acid and ezetimibe) tablets	Prevention of heart attacks and cardiovascular procedures in both primary and secondary prevention patients regardless of statin use	Approved for expanded population
Gilead	Vemlidy (tenofovir alafenamide)	Chronic hepatitis B virus infection in pediatric patients aged six and up weighing at least 55 lbs with compensated liver disease	Approved for expanded age indication
Abbott	TriClip transcatheter edge-to-edge repair (TEER) system	Tricuspid regurgitation	Approved
Medtronic	Evolut FX+ transcatheter aortic valve replacement (TAVR) system	Symptomatic severe aortic stenosis	Approved

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