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The CenterWatch Monthly

In This Issue

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Direct-to-Patient Shipment: Some Challenges Resolved, Some Remain

By James Miessler

After more than a decade of facing barriers, direct-to-patient (DTP) shipment of investigational products is coming into its own now that pandemic conditions have prompted solutions to major transportation problems. Sites and sponsors now have more DTP options, but there are still some kinks to work out and regulatory guidance needed.

The seeds of DTP were first sown in 2011 with Pfizer's fully virtual trial of Detrol LA (tolterodine tartrate), an overactive bladder treatment. Sites shipped the investigational drug to participants' homes instead of administering it at onsite visits. The trial spurred a new way of thinking about distributing investigational products, says Cat Hall, vice president of data and quality for Endpoint Clinical. But because it was unsuccessful, DTP was slow to be adopted, although it became well-known conceptually within the industry.

Serious initial barriers inherent to transporting investigational products and administering them at home contributed to apprehensions, Hall told *The CenterWatch Monthly*. These included the loss of cold chain control and risks to drug adherence away from the site. But as processes improved and technology advanced, some of these solutions have become great arguments in favor of DTP.

Temperature excursions — rises or drops in temperature outside a drug's allowed range — during transport used to mean returning the drug to the site or depot to assess its viability and creating new shipments. Now, wireless temperature monitors keep watch throughout a drug's journey and automatically sound the alarm on any issues. Often, issues are resolved before a driver knows they've occurred, Hall says.

Still, once the drug enters the patient's hands, it goes into a "Pandora's black box" of sorts, Hall explained. "Did they leave it in their hot car for an hour? Did they bring it home and put it in their bathroom, where there's high humidity, with the cap open?"

And it's hard to ensure the patient even took the drug or took it correctly without site staff there to observe. Addressing these issues will require new methods of supporting patient engagement, says R'Kes Starling, president and CEO of Reveles Clinical Services.

These could come in the form of automated reminders that "police" compliance or video apps that record the participant taking the drug.

"In a clinic, you have a direct observation. You can see it, you can give it to them, and in many cases, there's often an observational period where you assess

see **Direct-to-Patient** on page 4

Room for Change in Informed Consent? Addressing Length, Language and Reading Level

By Elizabeth Tilley Hinkle

Sites, sponsors and IRBs all agree that informed consent forms (ICF) need to be shorter, less technical and more readable for the lay person, but there can be tradeoffs in any kind of change. Explaining medical terms in plain language requires more words, adding to the length of the document. Removing legalese can make ICFs shorter but may leave a sponsor open to liability issues.

And while regulatory requirements and ethical principles limit what can be changed in ICFs, there are some creative approaches that can make the forms more understandable and less complex without sacrificing important content.

A recent study by the International Association for the Study of Lung Cancer (IASLC) highlights this decades-long discussion. The study involved a review of 20 ICFs and use of focus groups and a hypothetical ICF to discuss what information was most critical to people considering participation in a clinical trial. While the study focused primarily on non-small cell lung cancer trials, the issues raised apply equally to all areas of clinical research.

see **Room for Change** on page 5

Regulatory Update

FDA Calls for Loosening Eligibility in Noncurable Cancer Research

In an effort to broaden the eligibility criteria of noncurable cancer trials, the FDA has published final guidance recommending that sponsors and investigators allow patients who have not taken existing treatment options to participate.

The short guidance, which defines noncurative cancer as unresectable, locally advanced or metastatic disease in solid tumors or hematologic malignancies with unfavorable long-term overall survival, explains that patients who haven't received available therapy should be able to receive investigational drugs as long as they're adequately informed about their existing options prior to consenting.

To accommodate this group, the guidance offers three considerations for trial design. First, sponsors should ensure that all aspects of informed consent are addressed when consenting them, including alerting them to "appropriate alternative procedures or courses of treatment, if any, that might be advantageous."

In addition, patients who have and haven't received available therapies should be split into separate cohorts if interpreting the efficacy results requires a homogenous patient population. Efficacy analyses could also be done in prespecified subgroup analyses defined by prior receipt of available therapy.

Lastly, early dose-escalation trials may evaluate safety without considering participants' prior therapies as long as the drug's toxicities aren't expected to differ across patient subgroups, the agency said.

The draft form of the guidance was issued a year ago (CenterWatch, June 28, 2021).

Read the final guidance here: https://bit.ly/3OI7xjh.

EMA Sees Rise in Trial Applications Submitted to CTIS

The European Medicines Agency (EMA) has seen a modest but steady rise in the number of trial applications submitted to its new Clinical Trial Information System (CTIS) since the platform launched on Jan. 31.

Although most trial applications are still being filed to the EudraCT system, which stakeholders may continue using until Jan. 31, 2023, a recent EMA report shows that the number of applications filed to CTIS has risen month over month, starting with nine applications in February and rising to 18 in March, 29 in April, 35 in May and 39 in June. May and June each also saw one substantial modification submission.

By contrast, EudraCT application numbers are beginning to dip slightly; the system saw 193 applications in February, 200 in March, 187 in April, 178 in May and 175 in June.

So far, the agency has made 24 decisions on trial applications filed to CTIS, picking up speed as the year went on. It made just a single one in March, three in April and four in May, but shot up to 16 in June.

Read the full report here: https://bit.ly/3QiM3L9.

see **Regulatory Update** on page 3

Regulatory Update

continued from page 2

OIG Audit: NIH Dropped the Ball on Enforcing Trial Reporting Requirements

It's not just academia- and industry-funded trials that are lacking in transparency; a recent audit by the Office of Inspector General (OIG) has found that the National Institutes of Health (NIH) failed to ensure that the results of the trials it funded were posted on ClinicalTrials.gov in line with federal reporting requirements.

The audit, which reviewed 72 trials conducted or funded by NIH that are required by federal law and institute policy to post results, found that 25 of them did not comply with these requirements. In total, half (37) of these trials were noncompliant in some fashion.

In comparison, Oxford University's FDAAA tracker shows that, as of Aug. 17, approximately 3,500 (24 percent) of all clinical trials (federal, industry and private research) listed in CT.gov haven't posted their findings.

Among NIH trials, those conducted by researchers outside the institutes were found to be the least compliant, with one trial filing results late, 20 failing to submit results entirely and 15 posting results on time. Of trials conducted by NIH researchers, 11 submitted results late, five did not submit findings and 20 filed results on time.

OIG concluded NIH does not have adequate procedures for ensuring that responsible parties submit the results of clinical trials and that it "took limited enforcement action when there was noncompliance and continued to fund new research of responsible parties that had not submitted the results of their completed clinical trials."

In response to the OIG report, NIH agreed that it should improve its procedures, take enforcement action against responsible parties that submit results late or not at all, work with them to understand and identify the reporting hurdles they face, and develop procedures to address those challenges. It has already begun improving its internal procedures and activities to take action against negligent parties, NIH said.

Read the OIG report here: https://bit.ly/3dEppij.

View the FDAAA Tracker website here: https://bit.ly/2HEaAaQ.

Maryland Sponsor-Investigator Bypasses Trial Regulations, Earns FDA Ire

An herbal medicine specialist operating trials as a sponsor-investigator has been hit with an FDA Form 483 after a March inspection found she had skipped the investigational new drug (IND) approval, IRB review and informed consent processes in two trials for COVID-19 treatments.

Wanzhu Hou, who operates All Natural Medicine Clinic in Rockville, Md., failed to submit IND applications and receive FDA approval to conduct two trials that began in May and September of 2020, the agency's investigators found. These trials, which have enrolled 31 and 17 participants, last treated participants in early 2022 with natural medicine (combination products and dietary supplements) that the FDA considers to be investigational new drugs. They were still open to enrollment at the time of inspection, according to the 483.

In addition to not having the agency's green light, Hou did not submit written protocols and informed consent documents to an IRB for review. In fact, neither existed for the trials at the time of inspection.

By extension, the 483 also lists failure to obtain informed consent from each participant before administration of an investigational product.

There were also issues with recordkeeping in the two trials. Hou did not use any case report forms to document study data, with participant records being kept in an online electronic health record, and no records were kept related to participant eligibility. The trials also had no investigational drug accountability records to speak of. "Detailed records documenting shipment, receipt and disposition of investigational product were not maintained," the FDA said.

Access the Form 483 here: https://bit.ly/3AoGOmT.

Direct-to-Patient

continued from page 1

if there's adverse events or severe adverse events," he said. But when the patient takes the drug at home, "how will you manage the reporting and the collection of adverse events and things like that?" he posited. "It's going to require novel solutions that may or may not be fully integrated with the sites."

But DTP can also improve adherence, primarily by eliminating the risk of delayed site visits and resulting delays in taking medication. And it makes returning unused medications easier on both participants and the site. Many noncompliant participants avoid bringing their unused medicine back to the site, making it difficult to account for drug inventory and monitor adherence. DTP can easily be conducted in reverse, however, with couriers picking up leftovers from participants' homes and delivering them to the site.

While DTP shipment helps offer patients the greater flexibility they're growing to expect in trials, sites need to be aware that it can require more work on their side.

There are two mechanisms used for DTP, Hall says: depot-to-patient, where the drug moves directly from warehouse to patient, and site-to-patient, where the site receives the drug, stores it, interacts with a courier for pickup and performs other related tasks. It's this latter mechanism that can present sites with hassles; depot-to-patient, while not always possible, is ideal, although it too comes with important considerations.

"If things continue to move more toward depot-to-patient, then you've alleviated the site of all of that burden. There's less monitoring by clinical research associates, there's less oversight that they need to do, so I think the advantage, from a site perspective, is going depot-to-patient," she said. "However, at the same time, you want to be familiar with the drug and be able to give the patient instructions, and there's certain kinds of medications that just aren't viable that way, especially injectables."

From the sponsor point of view, the use of the depot approach can provide substantial cost savings because they aren't dedicating supplies to sites that might never enroll participants or may only have participants in the placebo group.

But Hall envisions a hybrid model in which sponsors provide sites with a small supply of the drug to be used for instruction and emergency replacements. A balance needs to be struck where the site has a small supply on hand but doesn't face the burden of receiving, storing and tracking the drug, as well as reporting temperature excursions and handling other responsibilities, she said.

And industry needs more guidance from the FDA, Hall said. Although the agency voiced its support for DTP and other decentralized approaches during the pandemic, now that the approach is becoming more widely used, the FDA needs to recognize it as a part of the trial landscape by providing specific direction. "I think there are still a lot of pathways to go for health authorities to get them to see that this is a long-term ongoing solution and provide better guidelines on what's expected," she said.

For example, DTP can be hampered by the differences among states' pharmacy board laws. The same is true of EU countries, she pointed out. Hall was part of conversations with the FDA on this very subject just before the pandemic hit, and it's an issue that the agency still needs to address, she said. State pharmacy boards, for instance, don't always understand that investigational products are excluded from transport restrictions, making interstate transportation problematic.

Any guidance the FDA develops should clearly define responsibilities and accountability in a DTP-enabled trial, says Jimmy Bechtel, vice president of site engagement for the Society of Clinical Research Sites.

Sites "are unsure how this will shift responsibility and what they are held accountable for," he said. "There needs to be an understanding and clarity on the role the site plays here and how responsibility shifts or changes. That is the big thing — who then becomes responsible for what."

Currently, oversight is the biggest concern for sites when it comes to DTP, according to Bechtel, because the principal investigator is still named as the person prescribing, assigning and/or dispensing the investigational product and holds ultimate responsibility for it unless this responsibility is delegated elsewhere. The compliance issues surrounding DTP can also become a serious safety concern, he noted.

"Several considerations need to be made around delegation, responsibility, liability, and if the patients can safely be brought into a trial like this," he said.

Room for Change

continued from page 1

Not surprisingly, the study concluded that ICFs are overwhelming due to their length, the language used and the amount and type of information provided.

There is general agreement within the clinical research world about what a well-designed consent form should look like. Three key features are expected:

- The document must contain benefit, risk and other information — some of which is mandated by regulation — to allow a patient to make an informed decision about participating in a clinical trial;
- It must be written at an appropriate reading level, generally agreed to be at the U.S. sixth- to eighth-grade level; and
- It must be of a length that enables patients to read it completely and thoroughly.

In other words, the goal is to have consent forms that have sufficient detail, are readable by the average patient and are relatively short, perhaps around 1,000 words, Stefan Grant, associate director for clinical and translational research and intellectual property development at Wake Forest Baptist Comprehensive Cancer Center, said.

"But this goal is nearly impossible to reach," Grant told *The CenterWatch Monthly*. "You can have any two of those three characteristics, but you cannot have all of them."

Forms in the IASLC study, for instance, ranged between 15 and 34 pages, with an average length of 21 pages. The study noted that readability varied by section but came in at an average reading level of tenth grade.

Grant placed much of the blame for the length and complexity of the typical ICF on regulations that specify certain boilerplate information that must be included. The list of mandatory items alone could take up 25 to 30 percent of a 1,000- to 1,250-word document, he said.

To reduce the length of ICFs, someone would need to look at the regulations and consider what is really necessary to include. Without that change, perhaps with the proviso that there must be opportunity for the patient to access additional data, Grant said, problems with the length of the ICF will be difficult to resolve.

Bellinda King-Kallimanis, director of patient-focused research at the LUNGevity Foundation, however, said that all parties are complicit in the problems. Rather than blaming drug companies, IRB committees or researchers, it's important to remember that "each of us has the ability to push back to get these forms more readable and accessible to patients and caregivers."

Sponsor concern about liability, for instance, can add legal information that lengthens ICFs without contributing to a person's ability to make a decision about trial participation.

A lot of the ICF is written by legal teams or from a lawyerly perspective, with language framed to avoid liability, Grant acknowledged. While pharma sponsors want to prevent liability by making sure that there is full disclosure of all possible risks, the goal of the clinical researcher is to ensure that study participants fully understand the risks and benefits; it can be challenging to balance these two goals.

For instance, Grant said, in studies of new indications for already-approved drugs, it's not uncommon for the risk portion of the consent form to list every side effect from the product's current labeling, in addition to any specific to the indication and target patient population for the clinical trial itself.

Some companies are more conservative in their approach to ICFs than others, and that is going to be difficult to control or influence, King-Kallimanis said. A better approach to streamlining ICFs would be to create an addendum containing key pieces of information that patients have indicated are most important to their decision.

Participants in the IASLC focus group did report being overwhelmed by the hypothetical ICF used for discussion purposes, which was based on the forms reviewed for the study. In particular, they reported that it was difficult to figure out what information was most important to their decision about participating in a study. Patients also said the forms appeared intended as anti-liability documents for the study sponsors.

And while patients participating in the focus group reported that they felt a longer ICF meant that important information was included, they also found that information too dense and difficult to understand, King-Kallimanis said.

"We don't really think there is a magic number of pages that will make or break someone's ability to feel informed about a clinical trial," King-Kallimanis said. "It's all about how the information is presented."

Attention also needs to be paid to readability. The average person reads at a sixth- to eighth-grade level, but ICFs contain a great deal of technical medical language that does not lend itself to presentation even at an eighth-grade reading level, much less to further simplification for less reading-proficient patients.

"Part of that is the challenge of having to explain complex concepts to people without a background in that area," Grant said. "For instance, if a patient is consenting to a trial that involves an antibody treatment, it can take a whole

see **Room for Change** on page 6

Room for Change

continued from page 5

paragraph to explain what an antibody is. And then the question arises as to whether someone needs that information in order to consent and be informed."

The answer to both the length and language challenges, King-Kallimanis said, could be found in additional, patient-focused documents. For example, the IASLC report suggested creation of a tri-fold brochure that summarizes key points of the ICF that are most relevant to participants. This could be provided as an addendum to the formal ICF and act as both a way to highlight the information most pertinent to clinical trial participation and present that information in more simple terms than the formal ICF.

Additionally, some simple reorganization of information in the ICF could prove helpful to patients' understanding. For example, the IASLC report suggested that an addendum to the ICF that provides a summary with references to specific page numbers in the document could help patients go back through the document to find and revisit particular pieces information.

"Another thing we did hear from patients that we interviewed was that putting all the contact information for different people on one page so that it is all together would be really helpful," King-Kallimanis said. "Sometimes, the information for the PI and then who to contact in case of an injury are not in the same sections. In a long document, patients didn't want to be flipping around looking for the right details."

Finally, it's important to remember that the ICF is not presented to the patient in a vacuum. Consenting is a process, not a piece of paper, Grant said. The discussion with a member of the research team is important, he added. Most people are not going to fully read and understand a long and technical document on their own.

And with all of the current concerns about the ICFs themselves, the one-on-one discussion with research staff becomes the most critical part for patient understanding, Grant said.

King-Kallimanis agreed about the value of both a useful ICF form and direct interaction with a member of the study team. The trust patients have with the team and the language used to describe the study are both important, and there is work needed in both areas, she said.

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		
Immunomic Therapeutics	ITI-3000	Merkel cell carcinoma
LG Chem	LG203003	Non-alcoholic steatohepatitis
I-Mab Biopharma	TJ-CD4B	Solid tumors, including gastric cancer, gastroesophageal junction carcinoma, esophageal adenocarcinoma and pancreatic ductal carcinoma
Blue Lake Biotechnology	Intranasal BLB-201 vaccine	Respiratory syncytial virus
ACM Biolabs	ACM-001 COVID-19 booster vaccine	COVID-19
Agenus	AGEN1571	Advanced solid tumors
Astria Therapeutics	STAR-0215	Hereditary angioedema
Bio-Thera Solutions	BAT8009	Advanced solid tumors
CytoImmune Therapeutics	CYTO-102	Relapsed/refractory non-small cell lung cancer
Ascletis Pharma	ASC61	Advanced solid tumors
Inmagene Biopharmaceuticals HutchMed	IMG-004	Inflammatory and autoimmune diseases
Innovent Biologics	IBI324	Diabetic macular edema
Innovent Biologics	IBI311	Active thyroid associated ophthalmopathy
Innovent Biologics	IBI363	Advanced solid tumors or lymphomas
BridgeBio Pharma	BBP-671	Propionic acidemia and methylmalonic acidemia
CureVac	CV0501 COVID-19 booster	COVID-19
Mersana Therapeutics	XMT-1660	Solid tumors including breast, endometrial and ovarian cancer
phase 1b		
Eledon Pharmaceuticals	Tegoprubart	Patients undergoing kidney transplantation
ADC Therapeutics	ADCT-601 (mipasetamab uzoptirine)	Advanced solid tumors
Upstream Bio	UPB-101	Asthma
Tessa Therapeutics	TT11 plus Opdivo (nivolumab)	Relapsed or refractory CD30-positive classical Hodgkin lymphoma

see **Study Lead Opportunities** on page 8

Study Lead Opportunities continued from page 7

Company name	Drug name	Indication
phase 1/2		
Siolta Therapeutics	STMC-103H	Prevention of atopic diseases in neonates and infants at risk for developing allergic disease
Ortho Regenerative Technologies	ORTHO-R	Rotator cuff tear repair
BioInvent	BI-1607 plus Herceptin (trastuzumab)	HER2+ solid tumors
Celularity	CYNK-101 plus trastuzumab and Keytruda (pembrolizumab)	Advanced HER2+ gastric and gastroesophageal junction cancer
Innovent Biologics Laekna Therapeutics	Afuresertib plus sintilimab plus chemotherapy	Solid tumors resistant to anti-PD-1/PD-L1 therapy
Tallac Therapeutics	TAC-001	Advanced solid tumors
Gamida Cell	GDA-201	Follicular and diffuse large B-cell lymphomas
Graphite Bio	GPH101 (nulabeglogene autogedtemcel)	Sickle cell disease
Passage Bio	PBFT02	Frontotemporal dementia with granulin mutations
phase 2		
ADC Therapeutics	Zynlonta (loncastuximab tesirine-lypl) in combination with rituximab	First-line diffuse large B-cell lymphoma
Cardior Pharmaceuticals	CDR132L	Heart failure after myocardial infarction
Cardurion Pharma	CRD-740	Heart failure
Daiichi Sankyo	DS-7300	Pretreated extensive-stage small cell lung cancer
George Medicines	GMRx4	Type 2 diabetes
MAIA Biotechnology	THIO	Advanced non-small cell lung cancer
Yuyu Pharmaceuticals	YP-P10	Dry eye disease
Celldex Therapeutics	Barzolvolimab	Chronic inducible urticaria
MediciNova	MN-001 (tipelukast)	Non-alcoholic fatty liver disease, type 2 diabetes mellitus and hypertriglyceridemia
Nanoscope Therapeutics	MCO-010	Stargardt disease
SOTIO Biotech	SOT101 plus Keytruda	Advanced/refractory solid tumors
Synaptogenix	Bryostatin	Alzheimer's disease
Cardiol Therapeutics	CardiolRx	Acute myocarditis
EyePoint Pharmaceuticals	EYP-1901	Maintenance treatment of wet age-related macular degeneration
OcuTerra Therapeutics	OTT166	Diabetic retinopathy
Artios	ART4215	BRCA-deficient breast cancer
Disc Medicine	Bitopertin	Erythropoietic protoporphyria or X-linked protoporphyria
Olema Oncology	OP-1250	ER+/HER2- metastatic breast cancer

see **Study Lead Opportunities** on page 9

Study Lead Opportunities continued from page 8

Company name	Drug name	Indication
phase 2 continued		
Saol Therapeutics	SL-1002	Knee pain associated with osteoarthritis
ALX Oncology	Evorpacept in combination with Erbitux (cetuximab) and Keytruda	Refractory microsatellite stable metastatic colorectal cancer in patients who have progressed on at least two lines of systemic therapy
Genexine	GX-188E, GX-I7 and nivolumab	Head and neck squamous cell carcinoma
phase 2a		
Impel Pharmaceuticals	INP105 (nasal olanzapine)	Acute agitation in adolescents with autism spectrum disorder
Neuraptive Therapeutics	NTX-001	Facial paralysis
Tarsus Pharmaceuticals	TP-03 (lotilaner ophthalmic solution)	Meibomian gland disease in patients with *Demodex* mites
phase 2b		
Sling Therapeutics	Linsitinib	Active moderate-to-severe thyroid eye disease
Denovo Biopharma	DB104 (liafensine)	Treatment-resistant depression
Union Therapeutics	Orismilast MR tablet	Moderate-to-severe atopic dermatitis
Noema Pharma	Gemlapodect (NOE-105)	Adults with childhood onset fluency disorder
phase 2/3		
Novavax	NVX-CoV2373 COVID-19 vaccine	COVID-19 in patients aged six months through 11 years
phase 3		
Aytu BioPharma	Enzastaurin (AR-101)	COL3A1-positive vascular Ehlers-Danlos syndrome
Capricor Therapeutics	CAP-1002	Late-stage Duchenne muscular dystrophy
Palisade Bio	LB1148	Return of bowel function in adults following gastrointestinal surgery
Aerie Pharmaceuticals	AR-15512	Dry eye disease
Avalo Therapeutics	AVTX-803	Leukocyte adhesion deficiency type II
NewAmsterdam Pharma	Obicetrapib	Heterozygous familial hypercholesterolemia
Orca Biosystems	Orca-T	Acute myeloid leukemia, acute lymphocytic leukemia and high-risk myelodysplastic syndromes
Sound Pharmaceuticals	SPI-1005	Meniere's disease
Daiichi Sankyo	Patritumab deruxtecan (HER3-DXd)	EGFR-mutated locally advanced or metastatic non-squamous non-small cell lung cancer
Pfizer Valneva	VLA15 Lyme disease vaccine	Lyme disease
Greenwich LifeSciences	GLSI-100	HER2/neu positive breast cancer in patients with residual disease or high-risk pathologic complete response at surgery who completed neoadjuvant and postoperative adjuvant trastuzumab treatment

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
Escend Pharmaceuticals	ES-3000	Relapsed or refractory acute myeloid leukemia	IND approved
Sorrento Therapeutics	STI-1558	COVID-19	IND approved
Omega Therapeutics	OTX-2002	Hepatocellular carcinoma	IND approved
HOOKIPA Pharma	HB-300	Metastatic castration-resistant prostate cancer	IND approved
Obsidian Therapeutics	OBX-115	Solid tumors	IND approved
Ascletis Pharma	ASC10	Mild-to-moderate COVID-19	IND approved
Astria Therapeutics	STAR-0215	Hereditary angioedema	IND approved
Avenge Bio	AVB-001	Peritoneal malignancies	IND approved
Calidi Biotherapeutics	NeuroNova	Recurrent high-grade glioma	IND approved
Cellectis	UCART20x22	B-cell malignancies	IND approved
Eccogene	ECC4703	Non-alcoholic steatohepatitis and dyslipidemia	IND approved
ImmunAbs	IM-101	Autoimmune diseases	IND approved
OliX Pharmaceuticals	OLX10212	Age-related macular degeneration	IND approved
Daewoong Pharmaceutical	DWP213388	Autoimmune diseases	IND approved
Eledon Pharmaceuticals	Tegoprubart	Prevention of organ rejection in patients receiving a kidney transplant	IND approved
Cure Rare Disease	CRD-TMH-001 CRISPR therapy	Duchenne muscular dystrophy	IND approved
Immunis	IMM01-STEM	Muscle atrophy related to knee osteoarthritis	IND approved
Neurocrine Biosciences	NBI-1117568	Schizophrenia	IND approved
Lantern Pharmaceuticals	LP-300 in combination with chemotherapy	Advanced non-small cell lung cancer in patients who have never smoked	Approval for a phase 2 trial granted
Renovion	ARINA-1	Bronchiolitis obliterans syndrome in bilateral lung transplant patients	Study May Proceed letter issued for phase 3 trial
Therapeutic Solutions International	JadiCell Universal Donor Stem Cell	COVID-19 acute respiratory distress syndrome	Emergency IND approved
CereVasc	eShunt System	Communicating hydrocephalus following subarachnoid hemorrhage	IDE approved
bluebird bio	Zynteglo (betibeglogene autotemcel)	Beta thalassemia in adult and pediatric patients who require regular red blood cell transfusions	Approved
Arcutis Biotherapeutics	Zoryve (roflumilast) cream 0.3 percent	Plaque psoriasis in patients age 12 and over	Approved

see **FDA Actions** on page 11

FDA Actions continued from page 10

Company name	Drug name	Indication	FDA action
Marius Pharmaceuticals	Kyzatrex (testosterone undecanoate)	Hypogonadism	Approved
ilooda	Secret Duo	Monkeypox scarring	Approved
Azurity Pharmaceuticals	Zonisade (zonisamide oral suspension)	Partial seizures in patients 16 and older with epilepsy	Approved
Incyte	Opzelura (ruxolitinib) cream	Nonsegmental vitiligo in patients 12 and older	Approved
Roche	Xofluza (baloxavir marboxil)	Treatment of acute uncomplicated influenza in otherwise healthy children age five to less than 12 years who have been symptomatic for no more than 48 hours	Approved for expanded indication
Roche	Xofluza	Postexposure prophylaxis of influenza in children age five to less than 12 years after contact with someone with influenza	Approved for expanded indication
Daiichi Sankyo AstraZeneca	Enhertu (fam-trastuzumab deruxtecan-nxki)	HER2-low metastatic breast cancer	Approved for expanded indication
Orion Bayer	Nubeqa (darolutamide)	Metastatic hormone-sensitive prostate cancer	Approved for expanded indication
Vivus	Qsymia (phentermine and topiramate extended-release capsules) CIV	Obesity in adolescents 12to-17 years old	Approved for expanded age indication
GlaxoSmithKline	Benlysta (belimumab)	Children age five to 17 years with active lupus nephritis	Approved for expanded age indication
Allergan Aesthetics	Juvéderm Volux XC	Improvement of jawline definition in adults over age 21	Approved for new indication
Myovant Sciences Pfizer	Myfembree (relugolix, estradiol and norethindrone acetate)	Moderate-to-severe endometrial pain in premenopausal women	Approved for new indication
Daiichi Sankyo AstraZeneca	Enhertu (fam-trastuzumab deruxtecan-nxki)	Previously treated HER2-mutant metastatic non-small cell lung cancer	Approved for new indication
AcuFocus	IC-8 Apthera intraocular lens	Cataracts	Approved
Biotronik	Pulsar-18 T3 peripheral self-expanding stent system	Endovascular treatments	Approved

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