- 03.17.23 - Pfizer and Astellas Seek Expanded Label for Expensive Prostate Cancer Drug (biospace)
  - Pfizer and Astellas hope to add another indication for their jointly marketed prostate cancer drug, Xtandi (enzalutamide), after announcing positive topline Phase III results Thursday. Xtandi is already approved to treat three types of prostate cancer – metastatic and non-metastatic castration-resistant prostate cancer and metastatic castration-sensitive prostate cancer. The latest data show Xtandi plus leuprolide, a gonadotropin-releasing hormone agonist significantly improved metastasis-free survival (MFS) in men with non-metastatic castration-sensitive prostate cancer (nmCSPC) over placebo plus leuprolide.
- 03.16.23 - In reversal, Sarepta to face FDA's panel of outside experts on Duchenne gene therapy (endpts)
  - Sarepta now will have a public hearing with the FDA's panel of outside experts on its Duchenne muscular dystrophy gene therap. The announcement comes just two weeks after Sarepta said the FDA told the company it had no plans for an advisory committee meeting. A date has not been set for the adcomm meeting, but it will be held sometime before the FDA's deadline of May 29 to decide whether or not to grant the therapy accelerated approval. In an investor call, Sarepta CEO Doug Ingram emphasized that the decision was not related to any new data or analysis. The meeting, he said, will likely focus on the clinical trial's surrogate endpoint — expression of dystrophin, an enzyme that protects muscles when they expand and contract that people with Duchenne do not make functional versions of, leading their muscles to atrophy over time. The therapy, known as SRP-9001, delivers a gene that encodes for a shortened version of dystrophin and is meant to be a long-term treatment for the disease.
- 03.15.23 - Mirum Announces Label Expansion for LIVMARLI in the United States to Include Infants Three Months of Age and Older (medtechalert)
  - Mirum Pharmaceuticals today announced that the FDA has approved a reduction in age from one year to three months for LIVMARLI (maralixibat) oral solution for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS). The label expansion was based on data from the RISE study which characterized the safety and tolerability of LIVMARLI in infants under one year of age with ALGS.
- 03.13.23 - Calliditas plans to file for full approval for rare kidney disease drug following PhIII data (endpts)
  - Calliditas believes Phase III confirmatory data it released Sunday for its oral steroid to treat a rare kidney disease could be the final piece needed to get full approval from the FDA. The trial studied Nefecon, the biotech's delayed-release budesonide capsules marketed as Tarpeyo, on primary IgA nephropathy (IgAN) patients who were also on a RAS inhibitor therapy. Tarpeyo scored accelerated approval from the agency in December 2021 and conditional marketing authorization from the European Commission in July 2022. The drug demonstrated a "highly statistically significant benefit" over placebo in a two-year period with nine months of treatment studying the primary endpoint of estimated glomerular filtration rate, a measure of level of kidney function. This week's data are a second batch from this trial.
- 03.10.23 - AdComm backs lymphoma expansion for Roche drug (pharmamanufacturing)
  - An FDA advisory panel has voted in favor of expanding approval for Roche's (Genetentech) Polivy combination to allow the regime to be used a first-line treatment option for patents with diffuse large B-cell lymphoma (DLBCL). The U.S. FDA's Oncologic Drugs Advisory Committee voted 11 to 2 in favor of the clinical benefit of the phase 3 POLARIX study of Polivy in combination with R-CHP for people with previously untreated DLBCL. If the label expansion is approved, Polivy could be used in combination with Rituxan plus cyclophosphamide, doxorubicin and prednisone for the treatment of people with previously untreated DLBCL. DLBCL is an aggressive, hard-to-treat disease and the most common form of non-Hodgkin lymphoma in the U.S. According to Roche, Polivy is the first treatment in 20 years to show a significant and clinically meaningful improvement in progression-free survival over the standard of care for first-line DLBCL. However, the FDA's briefing document shared prior to the AdComm meeting expressed concerns over just how clinically meaningful the trial data was, pointing to a 'modest progression-free survival benefit."
- 03.10.23 - Acadia Pharmaceuticals Announces U.S. FDA Approval of DAYBUE™ (trofinetide) for the Treatment of Rett Syndrome in Adult and Pediatric Patients Two Years of Age and Older (PR)
  - Supported by results from the pivotal Phase 3 LAVENDER study evaluating the efficacy and safety of trofinetide versus placebo in 187 female patients with Rett syndrome five to 20 years of age. In the study, treatment with DAYBUE demonstrated statistically significant improvement compared to placebo on both co-primary efficacy endpoints, as measured by the change from baseline in Rett Syndrome Behaviour Questionnaire total score (p=0.018) and the Clinical Global Impression-Improvement scale score (p=0.003) at week 12. The RSBQ is a caregiver assessment that evaluates a range of symptoms of Rett syndrome including vocalizations, facial expressions, eye gaze, hand movements (or stereotypies), repetitive behaviors, breathing, night-time behaviors and mood. The CGI-I is a global physician assessment of whether a patient has improved or worsened. In the study, the most common side effects were diarrhea (82%) and vomiting (29%).

- 03.10.23 - Pfizer's Biohaven Bet Pays Off Again as Migraine Nasal Spray Wins Approval (biospace)
  - Zavzpret is a small molecule antagonist of the calcitonin gene-related peptide (CGRP) receptor, which is an emerging target for acute migraine relief, particularly for patients who have contraindications or poor response to triptan-based medications. Zavzpret is the first CGRP antagonist nasal spray for migraine with or without aura. Data from two pivotal Phase III trials supported Zavzpret's FDA bid, both of which showed significantly better efficacy relative to placebo. Pfizer's nasal spray induced statistically higher rates of pain freedom – defined as a decrease in headache pain from moderate-to-severe to none – and freedom from the most bothersome symptom two hours after dosing. Pfizer first gained rights to Zavzpret in May 2022, when it dropped $11.6 billion to acquire leading migraine player Biohaven. Under the terms of the agreement, Pfizer bought all of Biohaven's outstanding shares and paid all of its third-party debt.
- 03.09.23 - Palvella Therapeutics Announces Positive Topline Results from Phase 2 Study of QTORIN™ 3.9% Rapamycin Anhydrous Gel (QTORIN™ rapamycin) for the Treatment of Microcystic Lymphatic Malformations, a Serious, Rare Genetic Skin Disease with No FDA-approved Therapies (PR)
  - QTORIN™ rapamycin generally well-tolerated; no drug related severe adverse events and no observed rapamycin in systemic circulation. 100% of participants were either "Much Improved" or "Very Much Improved" as rated by the Clinician Global Impression of Change following 12-weeks of QTORIN™ rapamycin. End of Phase 2 meeting completed with U.S. Food and Drug Administration in February 2023; pending additional interactions with FDA, anticipate potential initiation of pivotal Phase 3 study in second half of 2023. FDA previously granted Fast Track Designation and Orphan Drug Designation to QTORIN™ rapamycin for Microcystic Lymphatic Malformations. QTORIN™ rapamycin has potential to become first therapy and standard of care for the estimated more than 30,000 individuals with Microcystic Lymphatic Malformations in U.S., if approved
- 03.09.23 - Hikma Expands Impact in Canada with Launches of New Sterile Injectable Medicines (medtechalert)
  - All of the medicines, which will be launched soon, represent the first or second generic versions of their molecules on the Canadian market and treat conditions across a variety of therapeutic areas – a meaningful expansion in access to medicines for Canadian patients. With these launches, Hikma now markets approximately 30 different sterile injectable medicines in Canada, with plans to launch up to 13 additional sterile injectable products in Canada this year. The company is now a top 10 player in the Canadian generic sterile injectables market and has a leading presence in North America. In the US, Hikma is a top three supplier by volume of generic injectables to US hospitals.
- 03.09.23 - AstraZeneca lung cancer drug meets survival endpoint in late-stage trial (PR)
  - AstraZeneca's EGFR inhibitor, Tagrisso, demonstrated strong overall survival benefit in a phase 3 trial for adjuvant treatment of patients with early-stage EGFR-mutated lung cancer, according to the drugmaker. AstraZeneca says the new data confirms Tagrisso's potential to extend patients' lives in early-stage disease. Tagrisso, which was given the green light by the FDA in 2017, is approved to treat early-stage lung cancer in more than 90 countries, including in the U.S., EU, China and Japan, and additional global regulatory reviews are ongoing. Tagrisso is also approved for the 1st-line treatment of patients with locally advanced or metastatic EGFRm NSCLC and for the treatment of locally advanced or metastatic EGFR T790M mutation-positive NSCLC.
- 03.09.23 - Molecular Templates Announces IND Acceptance by FDA for MT-8421 ETB Program Targeting CTLA-4 (PR)
  - Preclinical data from MT-8421 showed that in a transgenic mouse model expressing human CTLA-4 and bearing syngeneic subcutaneous tumors, MT-8421 treatment depleted immune suppressive Tregs in the TME but not in the periphery. MT-8421 was well tolerated in a non-human GLP primate toxicology study and achieved serum levels well-above projected IC50 concentrations for Tregs in the TME. MTEM expects to initiate a first-in-human phase I study with MT-8421 by mid-year 2023 at a starting dose of 32 mcg/kg.
- 03.09.23 - AusperBio Announces First-in-Human Dosing of AHB-137, an Antisense Oligonucleotide Drug Candidate for the Treatment of Chronic Hepatitis B (PR)
  - AusperBio is a clinical-stage biopharmaceutical company with operations in China and the USA, dedicated to the development of innovative, best-in-class therapeutics for curing HBV infections. The company's mission is to become a world leader in antiviral therapeutics. The company has developed a proprietary ASO technology platform, which enables effective and potent targeted therapies for liver diseases, with the potential for expansion into new target areas beyond the liver. The Phase 1 clinical trial is a randomized, double-blinded, placebo-controlled study to evaluate the safety, tolerability, pharmacokinetics of AHB-137 in healthy volunteers and initial efficacy in CHB patients (clinicaltrials.gov # NCT05717686). AHB-137 can trigger the degradation of all HBV RNA and has demonstrated potent reduction of HBsAg with favorable safety profile in preclinical studies. The detailed preclinical profiles of AHB-137 have been submitted for two presentations at an upcoming scientific congress in June.
- 03.08.23 - Inhibikase Therapeutics Announces FDA has Lifted the Full Clinical Hold on IkT-148009 in Multiple System Atrophy (PR)
  - The commay is developing protein kinase inhibitor therapeutics to modify the course of Parkinson's disease, Parkinson's-related disorders, and other diseases of the Abelson Tyrosine Kinases; today announced the U.S. FDA has lifted the full Clinical Hold on IkT-148009, the Company's c-Abl inhibitor, in Multiple System Atrophy (MSA) allowing the Company to proceed with its plans for a future Phase 2 clinical trial in MSA.

- 03.08.23 - FDA Accepts Biologics License Application for Proposed Denosumab Biosimilar (drugtopics)
  - Sandoz has announced that the FDA has accepted the BLA for a proposed biosimilar of denosumab (Prolia; Xgeva) for the treatment of osteoporosis in post-menopausal women (and in men at increased risk of fractures), skeletal-related complications in cancer that has spread to the bone, treatment-induced bone loss, giant cell tumor of the bone, and hypercalcemia of malignancy refractory to bisphosphonate therapy, according to a company press release. Denosumab is a human monoclonal antibody, created to bind to and inhibit the RANKL protein that activates osteoclasts. By decreasing their activity, the agent curtails bone loss and lowers the risk of fractures and other major bone conditions, per Sandoz.
- 03.08.23 - SpringWorks Therapeutics Announces New England Journal of Medicine Publication of Phase 3 DeFi Trial Evaluating Nirogacestat in Adults with Desmoid Tumors (PR)
  - Nirogacestat Treatment Significantly Improved Progression-Free Survival, Objective Response Rate and Key Patient Reported Outcomes. New Drug Application Under Review by the FDA with PDUFA Action Date of August 27, 2023. About the DeFi Trial DeFi is a global, randomized (1:1), double-blind, placebo-controlled Phase 3 trial evaluating the efficacy, safety and tolerability of nirogacestat in adult patients with progressing desmoid tumors. The double-blind phase of the study randomized 142 patients (nirogacestat, n=70; placebo n=72) to receive 150 mg of nirogacestat or placebo twice daily.
- 03.10.23 - Eisai projects $7B in sales by 2030 for new Alzheimer's drug (endpts)
  - Eisai and Biogen's newly approved Alzheimer's drug Leqembi (lecanemab) is looking to do what its amyloid-targeted predecessor, Biogen's Aduhelm (aducanumab), has yet to accomplish — hit blockbuster status. In a press conference yesterday, Eisai execs projected $7 billion (1 trillion yen) in Leqembi worldwide sales by 2030, assuming broader coverage from CMS, which has so far limited the use of any amyloid-targeted Alzheimer's drugs to clinical trials. The $7 billion estimate is based on the company's estimate that about 2.5 million people will be eligible for treatment by 2030, with prevalence in Asia/Latin America and China potentially reaching 70% of the global total.
- 03.07.23 - Shorla Oncology Announces U.S. FDA Approval of Nelarabine Injection for the Treatment of T-cell Leukemia (PR)
  - Shorla Oncology, a US-Ireland pharmaceutical company, today announced that the U.S. Food and Drug Administration (FDA) approved the company's oncology drug, Nelarabine Injection, for the treatment of T-cell Acute Lymphoblastic Leukemia (T-ALL) and T-cell Lymphoblastic Lymphoma (T-LBL). Nelarabine Injection provides patients with an alternative to a product that has historically been in shortage. This marks the first product approved in the US market for Shorla. In preparation for the immediate commercial launch of Nelarabine Injection, Shorla is working with its commercialization services partner, EVERSANA®, which it announced August 2021.
- 03.06.23 - FDA Approves Autoinjector Version of Neulasta Biosimilar (centerforbiosimilars)
  - The FDA approved the prefilled autoinjector version of Coherus Biosciences' pegfilgrastim biosimilar (Udenyca), which will allow for patients at risk of febrile neutropenia to administer a full dose of pegfilgrastim at home. Coherus Biosicences announced that the FDA approved a single-dose, prefilled autojector version of its pegfilgrastim biosimilar (Udenyca; pegfilgrastim-cbqv), for the treatment and prevention of febrile neutropenia, a common complication related to chemotherapy treatment. The autoinjector device will allow for patients to be administered pegfilgrastim the day after receiving chemotherapy, decreasing the risk of patients developing febrile neutropenia. The Udenyca autoinjector is triggered by push-on-skin activation, allowing for reliable and immediate delivery of a full pegfilgrastim dose.
- 03.06.23 - With PhII success, BridgeBio wades deeper into dwarfism drug debate (endpts)
  - BridgeBio's experimental drug designed to treat complications of achondroplasia, or dwarfism, greatly surpassed Wall Street expectations Monday morning in the final cohort of a Phase II trial, sending the company's stock climbing high. Infigratinib, an oral small molecule drug targeting the gene that causes dwarfism, induced roughly three-centimeters-per-year faster growth compared to baseline in 10 patients after six months, BridgeBio said Monday. The company has already started enrolling patients for a Phase III trial and will take this cohort's dose — 0.25 mg/kg — into the pivotal study, CEO Neil Kumar told Endpoints News.
- 03.02.23 - Amneal Announces U.S. FDA Filing Acceptance of Abbreviated New Drug Application for Naloxone Hydrochloride Nasal Spray, USP, 4mg (PR)
  - naloxone hydrochloride nasal spray, USP, 4mg, which is the generic version of Narcan® and is used in the treatment of a known or suspected opioid overdose emergency. "Naloxone hydrochloride nasal spray is a critical tool in addressing the opioid public health emergency across the United States," said Andy Boyer, Chief Commercial Officer, Amneal Generics. "We are well prepared to launch this product at a significant scale and substantially increase access to this life-saving medicine as we work to help combat this endemic crisis."