- 03.01.23 - FDA Approves First Therapy for Friedreich's Ataxia (Updated) (biospace)
  - Just in time for Rare Disease Day, patients with a rare neuromuscular disorder got their first medicine as the FDA approved Reata Pharmaceuticals' omaveloxolone. Reata claimed a Rare Pediatric Disease Priority Review voucher with the approval. The annual cost for Skyclarys is $370,000, the company announced. Symptoms, including unsteady posture, difficulty walking and scoliosis typically begin in childhood, with most patients succumbing to the disease by their mid-30s, often from cardiomyopathy. FA affects approximately 5,000 people in the U.S. alone. Skyclarys activates Nrf2, a transcription factor that helps resolve inflammation by restoring mitochondrial function, reducing oxidative stress and inhibiting pro-inflammatory signaling. Reata ultimately prevailed on the strength of a registrational, Phase II trial of 103 patients where treatment with Skyclarys led to "significant neurological function compared to placebo." Patients treated with Skyclarys had statistically significant lower modified Friedreich Ataxia Rating Scale (mFARS) scores compared to placebo. The mFARS scale, which monitors patient function, is the gold standard assessment tool for FA.
- 03.01.23 - Deciphera Pharmaceuticals Announces Completion of Enrollment for Pivotal Phase 3 MOTION Study of Vimseltinib in TGCT (PR)
  - Deciphera Pharmaceuticals, Inc. , a biopharmaceutical company focused on discovering, developing, and commercializing important new medicines to improve the lives of people with cancer, today announced the completion of enrollment in the pivotal Phase 3 MOTION study of vimseltinib in patients with tenosynovial giant cell tumor (TGCT) not amenable to surgery. Deciphera expects to report top-line results in the fourth quarter of 2023.
- 02.28.23 - Merck's Keytruda fails more prostate and lung cancer PhIII trials (endpts)
  - Merck's cancer drug behemoth, the anti-PD1 Keytruda, has failed two more Phase III trials in the hard-to-treat prostate cancer setting and in a certain lung cancer, per a Tuesday update. That marks more than half a dozen Phase III flops in the past 12 months for the drug, which won its first approval in 2014 and has generated dozens of billions in revenue for Merck, accounting for $20.9 billion in global sales last year. The drug, when combined with Pfizer and Astellas' Xtandi and androgen deprivation therapy, did not show improvement compared to placebo plus those two drugs on radiographic progression-free survival or overall survival at an interim analysis in certain prostate cancer patients. With that, Merck is discontinuing KEYNOTE-641, the drugmaker said Tuesday. It marks at least the fourth late-stage failure for Keytruda in various prostate cancer trials since last March. The latest comes in patients with metastatic castration-resistant prostate cancer.
- 02.27.23 - Maze Therapeutics Announces Positive Phase 1 Results from First-in-Human Trial Evaluating MZE001 as a Potential Oral Treatment for Pompe Disease (PR)
  - ZE001 Was Well Tolerated and Reduced Glycogen Accumulation in Blood Cells in Healthy Volunteers. Phase 1 Results Support Advancement into Phase 2 Trial in Patients with Pompe Disease, Expected to Initiate in 2023. Data Presented at WORLDSymposium 2023. MZE001 is an oral glycogen synthase (GYS1) inhibitor that aims to address Pompe disease by limiting disease-causing glycogen buildup. GYS1 is an enzyme responsible for glycogen production. MZE001 is currently being evaluated as a potential oral treatment for patients with Pompe disease, as well as other glycogen storage disorders.
- 02.27.23 - Evelo Biosciences Reports that EDP2939, its First Oral Extracellular Vesicle Product Candidate, Commenced Dosing in a Phase 2 Psoriasis Trial (PR)
  - Evelo Biosciences, Inc. (Nasdaq:EVLO), a clinical stage biotechnology company developing a novel platform of orally delivered anti-inflammatory medicines acting on the small intestinal axis, SINTAX, today announced that its first extracellular vesicle (EV) product candidate, EDP2939, has progressed to dosing in a Phase 2 psoriasis clinical trial after completing a safety and tolerability review from a first cohort of human volunteers. Safety and tolerability assessment of multiple ascending dose cohorts continues.
- 02.27.23 - SpringWorks Therapeutics Announces FDA Acceptance and Priority Review of New Drug Application for Nirogacestat for the Treatment of Adults with Desmoid Tumors (PR)
  - Nirogacestat, an investigational gamma secretase inhibitor, for the treatment of adults with desmoid tumors. The NDA was granted Priority Review and has been given a Prescription Drug User Fee Act (PDUFA) action date of August 27, 2023. The FDA's Priority Review designation is given to investigational medicines that treat a serious condition and offer significant improvements in safety or effectiveness. In addition, the FDA has stated that it is not currently planning to hold an advisory committee meeting to discuss the application. Should the drug win approval, it would be the company's first since being spun out of Pfizer five and a half years ago.
- 02.24.23 - Destiny Pharma Agreement with Sebela Pharmaceuticals for NTCD-M3 (PR)
  - Exclusive collaboration and co-development agreement for NTCD-M3 with Sebela Pharmaceuticals® worth up to $570m plus royalties. Partnership with Sebela will finance the future clinical development and commercialization costs of NTCD-M3 in North America. Destiny Pharma retains majority rights for Europe and ROW. Key strategic target achieved for NTCD-M3.

- 02.24.23 - Pfizer's Paxlovid gets March FDA advisory meeting to discuss full approval (endpts)
  - Pfizer's antiviral drug for Covid-19 is looking to inch closer to full FDA approval, with an agency advisory committee scheduled for March. The meeting is going to be held on March 16 from 9 a.m. to 5 p.m. EST, according to a document in the federal register from the US Department of Health and Human Services. The meeting will focus on whether Paxlovid should be approved to treat mild-to-moderate Covid-19 in adults who are at high risk for the virus' progression to a severe case which can lead to hospitalization or death. Currently the antiviral is approved only on an emergency-use basis.
- 02.22.23 - Amicus Therapeutics Announces Positive Long-Term Data from Phase 3 Open-label Extension Study of AT-GAA in Late-Onset Pompe Disease
  - Meaningful and Durable Responses in Key Endpoints of Six-Minute Walk, Forced Vital Capacity for ERT-Naïve and ERT-Experienced Participants Out to Two Years. Consistent Reduction in Biomarkers Continue to Suggest a Positive Effect on Muscle Tissue; Including Participants who Switched from alglucosidase alfa to AT-GAA in the Open-label Extension. Safety Profile Aligns with Previously Reported Data
- 02.21.23 - Vaxcyte Announces FDA Clearance of Investigational New Drug Application for VAX-24 for the Prevention of Invasive Pneumococcal Disease in Infants (PR)
  - Infant Phase 2 Study Initiation Expected in the Second Quarter of 2023, with Initial Topline Safety, Tolerability and Immunogenicity Data by 2025. Based on Positive Topline VAX-24 Phase 1/2 Proof-of-Concept Study Results in Adults, FDA Supported Initiation of Pediatric Program in Infants. Despite the effectiveness of current vaccines, IPD, which includes meningitis and bacteremia, remains persistent in the first years of life and is a leading cause of invasive disease in children two years of age and under. The burden of disease in the pediatric population underscores the need for a broader-spectrum vaccine.
- 02.18.23 - Apellis' Syfovre Nets First FDA Approval For Geographic Atrophy (geneonline)
  - Apellis Pharmaceuticals has announced the US FDA approval of Syfovre (pegcetacoplan injection) to treat geographic atrophy (GA), an advanced form of age-related macular degeneration (AMD). Syfovre makes history as the first and only FDA-approved treatment for GA, a leading cause of blindness that affects more than one million people in the US and five million people worldwide. The drug's approval for GA covers patients with or without subfoveal involvement and allows for a flexible dosing regimen of every 25 to 60 days.While Syfovre is designed to slow the progression of GA, it does not reverse the atrophy. The approval of the drug is based on positive two-year results from the Phase 3 Oaks and Derby studies, which showed that treatment reduced the rate of GA lesion growth compared to placebo. Increasing treatment effects were observed over time, with the greatest benefit (in Derby, 36% reduction in lesion growth with monthly treatment) occurring between months 18-24.
- 02.28.23 - Sutro Biopharma Appoints Dr. Anne Borgman as Chief Medical Officer (PR)
  - Prior to joining Sutro, Dr. Borgman served as Vice President and Therapeutic Area Lead, Oncology, Hematology, and Transplant, at Jazz Pharmaceuticals, where she was responsible for global drug development for four marketed products and drug development plans for several emerging targets. Previously, Dr. Borgman was Vice President, Clinical Research & Development, at Exelixis, where she was responsible for the global development for cabozantinib and oversaw the development of multiple Phase 3 programs. She has also held leadership positions in Oncology Drug Development at KaloBios Pharmaceuticals, Talon Therapeutics (formerly Hana Biosciences), and Abbott Laboratories. Dr. Borgman currently serves on the Board of Directors at Curis, NextCure, and NiKang Therapeutics and has been a Consulting Associate Professor for the Stanford University School of Medicine and at the University of Chicago.
- 02.22.23 - Gilead Presents Positive Proof-of-Concept Data for Investigational Combination Regimen of Lenacapavir with Broadly Neutralizing Antibodies as a Potential Twice-Yearly Approach for the Treatment of HIV (PR)
  - Study Demonstrates the Potential of Lenacapavir in Combination with Broadly Neutralizing HIV Antibodies Teropavimab and Zinlirvimab. Findings Support Further Evaluation of the Investigational Combination as a Long-Acting HIV Treatment Option in a Phase 2 Study. The combination of lenacapavir with teropavimab and zinlirvimab will advance to a Phase 2 study (NCT05729568) later this year in virologically suppressed people living with HIV. The study will assess two different dose levels of the bNAbs and assess safety and efficacy of the regimen in participants followed longitudinally for multiple doses of the study regimen.
- 02.16.23 - FDA gives green light to Chiesi's Lamzede (pharmaphorum)
  - US regulators have approved Chiesi's Lamzede (velmanase alfa-tycv) as the first enzyme replacement therapy for the treatment of non-central nervous system manifestations of alpha-mannosidosis (AM) in adult and paediatric patients. Lamzede (velmanase alfa-tycv) is a recombinant form of human alpha-mannosidase designed to replace or support the function of the natural enzyme and prevent the build-up of mannose–rich oligosaccharides in various tissues in the body.
- 02.16.23 - Inozyme Pharma Reports Positive Topline Data from Ongoing Phase 1/2 Trials of INZ-701 (PR)
  - Rapid, significant, and sustained increase in plasma pyrophosphate (PPi) observed and encouraging patient reported outcome data in all dose cohorts in ENPP1 Deficiency trial. Rapid and significant increase in PPi observed in all dose cohorts with sustained increase observed in highest dose cohort in ABCC6 Deficiency.(PXE) trial - INZ-701 was generally well-tolerated and exhibited a favorable safety profile in both trials.

- 02.15.23 - Coherus and Junshi Biosciences Announce Positive Final Overall Survival Results of JUPITER-02, a Phase 3 Clinical Trial Evaluating Toripalimab as Treatment for Recurrent or Metastatic Nasopharyngeal Carcinoma (PR)
  - The FDA has granted Breakthrough Therapy designations and priority review for the toripalimab Biologics License Application ("BLA") for use in combination with gemcitabine and cisplatin as first-line treatment for patients with advanced recurrent or metastatic NPC and for toripalimab monotherapy for the second-line or later treatment of recurrent or metastatic NPC after platinum-containing chemotherapy. Recurrent or metastatic NPC is an aggressive head and neck tumor which has no FDA-approved treatment options.
- 02.14.23 - Kinnate Biopharma Inc. Receives Fast Track Designation from the U.S. Food and Drug Administration for KIN-3248, an Investigational Pan-FGFR Inhibitor (PR)
  - Fast Track designation for Kinnate's investigational pan-FGFR inhibitor, KIN-3248, for the treatment of patients with unresectable, locally advanced or metastatic cholangiocarcinoma (CCA) harboring fibroblast growth factor receptor 2 (FGFR2) gene fusions or other alterations, who have received at least one prior systemic therapy. Cholangiocarcinoma, also known as bile duct cancer, is a rare condition, often diagnosed when it is advanced. Research has shown that FGFR is an actionable alteration in patients with CCA. FGFRs are tyrosine kinases that play a crucial role in cell proliferation, differentiation, migration and survival. FGFR2 gene fusions or other alterations are identified in approximately 16% of intrahepatic cholangiocarcinoma (ICC) tumors.
- 02.13.23 - Exelixis and Sairopa Announce US FDA Clears Investigational New Drug Application for ADU-1805 in Patients with Advanced Solid Tumors (PR)
  - Under the terms of the clinical development and option agreement announced in November 2022, Exelixis has the option to obtain an exclusive, worldwide license to develop and commercialize ADU-1805 and other anti-SIRPα antibodies upon review of data from prespecified phase 1 clinical studies of ADU-1805 to be completed by Sairopa during the option period. This IND clearance triggers a $35 million milestone payment to Sairopa which will be paid in the first quarter of 2023.
- 02.13.23 - Opdivo® (nivolumab) in Combination with CABOMETYX® (cabozantinib) Shows Durable Survival with Over Three Years of Follow-Up in the CheckMate -9ER Trial in First-Line Advanced Renal Cell Carcinoma (PR)
  - Data to be featured at ASCO GU 2023 demonstrate continued overall survival, progression-free survival and objective response rate benefits with Opdivo in combination with CABOMETYX compared to sunitinib, regardless of IMDC risk score. These three-year data – with a median follow-up of 44 months – from CheckMate -9ER represent the longest reported follow-up in any Phase 3 trial with an immunotherapy-tyrosine kinase inhibitor regimen in this population. In an exploratory biomarker analysis, median progression-free survival and overall survival were improved with the combination of Opdivo and CABOMETYX regardless of PD-L1 status
- 02.10.23 - Incyte Announces 52-Week Results from Phase 2 Study Evaluating Povorcitinib (INCB54707) in Patients with Hidradenitis Suppurativa (PR)
  - Results from open-label extension period of the Phase 2 trial demonstrate that longer-term treatment with povorcitinib 75 mg resulted in sustained and durable efficacy across all treatment arms. Data featured as an oral presentation at the European Hidradenitis Suppurativa Foundation conference. Hidradenitis suppurativa (HS) is a chronic and debilitating inflammatory skin condition characterized by painful nodules and abscesses that can lead to irreversible tissue destruction and scarring
- 02.10.23 - Mineralys, amid signs of thawing IPO market, upsizes offering to raise $192M for race with AstraZeneca (fiercebiotech)
  - In the first half of 2023, Mineralys plans to start a phase 2 clinical trial to evaluate its lead candidate, the aldosterone synthase inhibitor lorundrostat, as an add-on drug in patients with uncontrolled or resistant hypertension (uHTN/rHTN). It also expects to kick off another phase 2 trial of lorundrostat for the treatment of uHTN and rHTN in a chronic kidney disease population around the midpoint of the year.