CITELINE COMMERCIAL

Report

Q2 2023 Outlook Report

March 2023

Brought to you by:

BIOMEDTRACKER
CITELINE COMMERCIAL

MEDDEVICETRACKER
CITELINE COMMERCIAL

Contents

About the Author

Biomedtracker is an independent research service that offers proprietary clinical assessments and patient-based revenue forecasts of developmental drugs within a comprehensive and intuitive drug information database. Clients from the pharmaceutical, biotech, and investment industries rely on Biomedtracker for its insight on the likelihood of approval, commercial potential, and future data and regulatory catalysts for drugs within the competitive landscape of every important disease and indication. Over the last several years, Biomedtracker has become the leader in providing objective information alongside evidence based clinical assessments and investment research on pipeline drugs worldwide. For more information on getting direct access to Biomedtracker, please email BiomedSupport@sagientresearch.com.

Executive Summary

In this report, we cover catalysts from 24 drugs, devices, diagnostics, and deals expected to occur in Q2 2023. For each drug, the likelihood of Phase/PDUFA review success and overall Likelihood of Approval (LOA) given their particular phase, drug class, and disease group are provided. These data points were provided using a combination of Pharmapremia, our drug development benchmarking product utilizing Biomedtracker's LOA data to assist in informed decisions about drug pipeline prioritization, partnerships, and acquisitions, and drug approval data from Biomedtracker. The results of the catalysts highlighted in our Early 2023 Outlook Report can be found on Page 4. At the end of this report, we have included a list of Large Impact catalysts through Q2 2023. The catalyst list is also provided in Excel by downloading the supplemental material at the top of this page. Like our report? Have any questions or feedback? Please let us know at askanalyst@sagientresearch.com.

Disclaimer

This report is published by Sagient Research (the Publisher). This report contains information from reputable sources and although reasonable efforts have been made to publish accurate information, you assume sole responsibility for the selection, suitability and use of this report and acknowledge that the Publisher makes no warranties (either express or implied) as to, nor accepts liability for, the accuracy or fitness for a particular purpose of the information or advice contained herein. The Publisher wishes to make it clear that any views or opinions expressed in this report by individual authors or contributors are their personal views and opinions and do not necessarily reflect the views/opinions of the Publisher.

Outcomes of Biomedtracker's Large Impact Drug Catalysts from the Early 2023 Outlook Report							
Occurred Date	**Lead Company**	**Product**	**Market**	**Catalyst**	**Did LOA Predict Outcome**	**LOA Before Outcome**	**LOA After Outcome**
01/06/2023	Eisai Co.	Leqembi	Neurology	PDUFA for BLA - First Review (Accelerated Approval)	Yes	95% (5% Above Avg.)	100% (Same As Avg.)
01/19/2023	Eli Lilly	Donanemab	Neurology	PDUFA for BLA - First Review	No	90% (3% Above Avg.)	49% (3% Above Avg.)
01/10/2023	AstraZeneca	AIRSUPRA	Respiratory	PDUFA for NDA - First Review	Yes	99% (4% Above Avg.)	100% (Same As Avg.)
01/30/2023	The Menarini Group	Orserdu	Oncology	PDUFA for NDA - First Review	Yes	97% (5% Above Avg.)	100% (Same As Avg.)
09/16/2021	MacroGenics	Margenza	Oncology	Phase II/III - MAHOGANY - Cohort A - Top-Line Results at ESMO	Yes	46% (2% Above Avg.)	46% (2% Above Avg.)
02/09/2023	Phathom Pharmaceuticals	Takecab	Autoimmune/immunology	PDUFA for NDA - First Review	No	99% (5% Above Avg.)	99% (5% Above Avg.)
03/22/2023	Melinta Therapeutics	REZZAYO	Infectious Disease	PDUFA for NDA - First Review	Yes	99% (6% Above Avg.)	100% (Same As Avg.)
02/23/2023	Sanofi	Altuviiio	Hematology	PDUFA for BLA - First Review	No	99% (6% Above Avg.)	100% (Same As Avg.)
01/27/2023	Eli Lilly	Jaypirca	Oncology	PDUFA for NDA - First Review	Yes	96% (4% Above Avg.)	100% (Same As Avg.)
03/10/2023	Pfizer	Zavzpret	Neurology	PDUFA for NDA - First Review	Yes	98% (11% Above Avg.)	100% (Same As Avg.)
03/24/2023	Pharming Group	Joenja	Autoimmune/immunology	PDUFA for NDA - First Review	Yes	99% (5% Above Avg.)	100% (Same As Avg.)
03/10/2023	ACADIA Pharmaceuticals	Daybue	Neurology	PDUFA for NDA - First Review	No	92% (5% Above Avg.)	100% (Same As Avg.)

[1]No prior LOA change

Drugs

SUL-DUR for Acinetobacter-Specific Agents (Antibacterial)

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
SUL-DUR	Innoviva, Inc.	Zai Lab	Acinetobacter-Specific Agents (Antibacterial)	5/29/2023	PDUFA for NDA - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
NDA	Infectious Diseases	NME	87.0%	85.0%	**Above**

SUL-DUR, a combination therapy comprising of two antibiotics, Sulbactam and Durlobactam, is being developed by Innoviva for the treatment of infections caused by the Acinetobacter baumannii-calcoaceticus complex (ABC), including multi-drug resistant and carbapenem-resistant strains.

There is an urgent need for effective treatments for these infections, as they are associated with high morbidity and mortality rates, and the Acinetobacter species are innately resistant to many classes of antibiotics, including penicillin, chloramphenicol, and often aminoglycosides. ABC infections are a significant public health concern, particularly among immunocompromised individuals and those hospitalized for prolonged periods. The drug's Fast Track and Qualified Infectious Disease Product (QIDP) designations assigned to SUL-DUR reflect the urgent need for new therapies to treat these infections.

SUL-DUR has shown promising results in preclinical and clinical studies, demonstrating potent activity against ABC infections, including those caused by multi-drug resistant and carbapenem-resistant strains. The pivotal Phase III ATTACK trial evaluating the safety and efficacy of SUL-DUR in the treatment of 465 patients with infections caused by ABC showed that SUL-DUR met the primary endpoint of non-inferiority compared to imipenem/cilastatin, a commonly used antibiotic for the treatment. Another more noticeable result was that twice as many deaths occurred in the colistin arm than in the SUL-DUR arm through day 28. SUL-DUR also demonstrated a favourable safety profile, with a similar incidence of adverse events compared with colistin.

SUL-DUR addresses an unmet medical need, as there are currently limited treatment options for ABC infections, particularly those caused by multi-drug resistant and carbapenem-resistant strains. The U.S. Food and Drug Administration’s (FDA) Antimicrobial Drugs Advisory Committee will convene April 17, 2023 to review data supporting the new drug application (NDA) for SUL-DUR which was filed in September 2022. As this filing received a priority review voucher in November 2022, the Prescription Drug User Fee Act (PDUFA) is currently set for May 29, 2023.

Bylvay for Alagille Syndrome

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
Bylvay	Ipsen SA	Gen Ilac, Genpharm, Jadeite Medicines, Medison, Travere Therapeutics	Alagille Syndrome	06/15/2023	PDUFA for sNDA - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
NDA	Metabolic	NME	79.5%	95.0%	**Above**

Bylvay, odevixibat, is an inhibitor of the ileal bile acid transporter (IBAT), decreasing the re-absorption of bile acids into the distal part of the small bowel in order to reduce abnormal levels of bile acids. The drug was developed by Albireo, which was acquired by Ipsen in March 2023. It is already approved for pruritus in all subtypes of progressive familial intrahepatic cholestasis (PFIC), but is under development for pruritus from Alagille syndrome, a condition in which bile builds up in the liver because there are too few bile ducts to drain the bile. Similar to PFIC, the drug has an Orphan Drug Designation for Alagille syndrome.

The pivotal Phase III ASSERT trial met the primary endpoint showing statistically significant reduction in pruritus as measured by the PRUCISION Observer-Reported Outcome scratching score (0-4 point scale), from baseline at month 6 (weeks 21 to 24), compared to the placebo arm (p=0.002). The difference from placebo was not numerically quite as large as that in the label of approved IBAT inhibitor Livmarli, but that was a randomized withdrawal trial and differences in designs and patients make it difficult to compare the results. Odevixibat's study correspondingly showed a statistically significant reduction in serum bile acid concentration from baseline to the average of weeks 20 and 24 (compared to the placebo arm p=0.001). Odevixibat was also well tolerated, though patients in its trial arm did have a slight increase in diarrhea (11.4% versus 5.9% placebo) and vomiting (5.7% versus 0%), but no discontinuations due to AEs.

Albireo has reported that the FDA is only requiring a single successful Phase III study for approval. The Prescription Drug User Act (PDUFA) action date is assigned for June 15, 2023, where an approval would be an important line extension, increasing the drug's potential patient pool by over 2.5 fold per company estimates. The drug of course threatens Livmarli, the only currently approved Alagille syndrome treatment.

Ritlecitinib for Alopecia Areata

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
Ritlecitinib	Pfizer, Inc.	N/A	Alopecia Areata	4/1/23 - 6/30/23	PDUFA for NDA - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
NDA	Dermatology	New Molecular Entity (NME)	99.0%	89.0%	**Above**

Ritlecitinib is a small molecule JAK3 inhibitor under development by Pfizer for the treatment of alopecia areata. The drug received Breakthrough Therapy Designation in the U.S. for the indication in September 2018. As of June 2022, Pfizer has submitted ritlecitinib for approval in the U.S., Europe, the U.K., Japan and China based on the results of their global Phase IIb/III ALLEGRO study.

The pivotal Phase IIb/III ALLEGRA study was initiated in 2019 and evaluated the efficacy and safety of ritlecitinib in 719 adult and adolescent alopecia areata subjects with 50% or greater scalp hair loss. Patients were randomized to receive ritlecitinib 50 mg or 30 mg (with or without one month of initial loading dose treatment of once-daily ritlecitinib 200 mg), ritlecitinib 10 mg or placebo. Topline results released in August 2021 showed that both ritlecitinib 50 mg and 30 mg achieved the primary efficacy endpoint of proportion of patients with scalp hair regrowth in response to ritlecitinib treatment, based on an absolute Severity of Alopecia Tool (SALT) Score ≤20 at Week 24.

These positive data put ritlecitinib in the running to potentially compete with Eli Lilly's Olumiant, a JAK1/2 inhibitor which has demonstrated around a 30% placebo-adjusted improvement in the proportion of patients reaching 80% or greater scalp hair coverage after 36 weeks of 4mg/day treatment. Like all other JAK inhibitors on the market, Olumiant's label carries a black box warning for serious infections, lymphoma, and thrombosis, which could be a potential barrier to uptake. Although in the ALLEGRO trial, the most common adverse events observed with ritlecitinib treatment were nasopharyngitis, headache, and upper respiratory infection, with no major adverse cardiac events, deaths, or opportunistic infections, ritlecitinib may also have this safety warning if it is approved, as this drug falls into the same class as Olumiant.

Regulatory decisions are ongoing in the U.K., Japan and China. Both the EMA in Europe and the FDA in the United States accepted the New Drug Application (NDA) filing for ritlecitinib for adults and adolescents 12 years of age and older with alopecia areata in September 2022. The FDA is expected to make a decision in the second quarter of 2023 and the EMA in the fourth quarter of 2023.

Tofersen for Amyotrophic Lateral Sclerosis (ALS)

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
Tofersen	Biogen, Inc.	Ionis Pharmaceuticals, Inc.	Alzheimer's Disease (AD)	4/25/2023	PDUFA for NDA - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
NDA	Neurology	New Molecular Entity (NME)	87.3%	64.0%	**Above**

Tofersen (BIIB067), which Biogen licensed from Ionis Pharmaceuticals for the treatment of superoxide dismutase 1 (SOD1) amyotrophic lateral sclerosis (ALS), is set to receive an approval decision from the United States Food and Drug Administration (FDA) on April 25, 2023. SOD1 ALS is a rare, fatal, neurodegenerative disorder caused by mutations in the SOD1 gene leading to progressive loss of motor neurons. Mutations in the gene for SOD1 have been associated with about 20% of cases of familial ALS and familial ALS represents about 10% of ALS cases. Tofersen is an antisense oligonucleotide that mediates the degradation of SOD1 mRNA to reduce SOD1 protein synthesis. Currently, there are no disease-modifying treatments for available for SOD1 ALS, making tofersen's approval a potentially significant breakthrough for ALS patients.

Tofersen has been evaluated in a number of clinical trials over the last decade, including a Phase I/II trial initiated in 2015 that demonstrated safety and tolerability, as well as a reduction in SOD1 protein levels in the cerebrospinal fluid of participants. Disappointingly, VALOR, a six-month pivotal Phase III study in 108 participants, did not meet its primary endpoint of change from baseline to week 28 in the Revised Amyotrophic Lateral Sclerosis Functional Rating Scale. However, 95 of these patients enrolled in the open-label extension (OLE) study, and combined VALOR and OLE 12-month data show sustained reductions in SOD1 protein (a marker of target engagement) and neurofilament (a marker of neurodegeneration).

The New Drug Application (NDA) for tofersen was granted priority review in July 2022 and includes data from a Phase I in health volunteers, the Phase I/II study evaluating ascending dose levels, the Phase III VALOR study, and the open-label extension (OLE) study. The FDA Peripheral and Central Nervous System Drugs Advisory Committee will convene March 22, 2023 to discuss the NDA for tofersen and, if approved, tofersen would be the first disease-modifying treatment for SOD1 ALS. Patients with SOD1 ALS typically have a shorter survival time than those with other forms of ALS, making the development of effective treatments a critical unmet need.

Omidubicel for Bone Marrow Transplant and Stem Cell Transplant

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
Omidubicel	Gamida Cell Ltd.	Lonza Group, Novartis	Bone Marrow Transplant and Stem Cell Transplant	05/01/2023	PDUFA for BLA - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
BLA	Oncology	Biologic	93.2%	90%	**Above**

Omidubicel is an umbilical cord blood (UCB)-derived ex Vivo expanded stem and progenitor cells treatment. The biologic is developed based on Nicotinamide (NAM) technology, a Vitamin B3, a potent modulator of SIRT1 activity as well as a potent inhibitor of NAD+ -dependent ADP ribosyl transferase enzymes. The drug is currently in clinical development for use in allogeneic hematopoietic (bone marrow) stem cell transplants for patients with hematologic malignancies, such as blood cancers. In October 2016, the US FDA granted omidubicel Breakthrough Therapy Designation for bone marrow transplant (BMT) in patients with high risk hematological malignancies such as leukemia and lymphoma, and Orphan Drug Designation as a treatment for hematopoietic stem cell transplant.

Gamida Cell has studied omidubicel in several clinical studies including a pivotal Phase III multicenter, randomized trial of transplantation of omidubicel, ex vivo expanded, UCB-derived, stem and progenitor cells, versus unmanipulated UCB for patients with hematological malignancies that was initiated in December 2016. Top-line results for this study were announced in May 2020 with the primary endpoint of time to neutrophil engraftment being achieved with statistical significance. In the intent-to-treat analysis, the median time to neutrophil engraftment was significantly shorter for patients who received omidubicel, achieving neutrophil engraftment at 12 days compared to the comparator group who reached neutrophil engraftment at 22 days. Later in 2020, updated results showed that the study also achieved all three secondary endpoints of the study which were proportion of patients who achieved platelet engraftment by day 42, the proportion of patients with grade 2 or grade 3 bacterial or invasive fungal infections in the first 100 days following transplant, and the number of days alive and out of the hospital in the first 100 days following transplant. Based on the positive and statistically significant results from this study, the company initiated a rolling BLA submission in the beginning of 2022 and completed the submission in June.

Upon interactions with the US FDA related to the BLA for omidubicel, the FDA issued an information request and viewed the data in the response as a major amendment, resulting in an extension of the PDUFA date from January 30, 2023 to May 1, 2023. The therapy appears to be both clinically and commercially attractive. The therapy appears to be both clinically and commercially attractive. Omidubicel not only provides a more ready alternative to mismatched traditional BMT donors with comparable efficacy but also meets a currently unmet need by increasing the speed of engraftment for patients receiving umbilical cord blood and therefore decreasing the risk of infection in immunocompromised patients. Omidubicel is one of three drugs currently under review by the FDA for stem cell transplant, and if approved, it will be one of five approved treatments in the US.

Trastuzumab Duocarmazine for Breast Cancer

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
Trastuzumab Duocarmazine	Byondis B.V.	medac	Breast Cancer	05/12/2023	PDUFA for BLA – First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
BLA	Oncology	Biologic	93.2%	90%	**Above**

Trastuzumab duocarmazine (SYD985) is a HER2-targeting antibody-drug conjugate (ADC) based on trastuzumab and Synthon's proprietary cleavable linker- duocarmycin (vc-seco-DUBA) payload. The duocarmycins are potent DNA minor groove binding alkylators attached to the antibody through a proprietary SpaceLink linker. Synthon uses fully synthetic duocarmycin prodrugs that are converted to the active form once released from the linker. Byondis announced that it submitted both a Biologics License Application (BLA) from the U.S. Food & Drug Administration (FDA) and a Marketing Authorization Application (MAA) from the European Medicines Agency (EMA) in July 2022 which are currently under review.

Results from the Phase III TULIP study (which began in 2017 and enrolled 437 female patients with a median age of 56 and a median of 4 prior metastatic breast cancer (MBC) treatments) showed significantly improved progression-free survival (PFS) in comparison with standard physician's choice (PC) treatment. These positive results may provide a new treatment option for patients with metastatic HER2-positive and pre-treated locally advanced MBC. The study met its primary endpoints, demonstrating that SYD985 is superior to some physician's choice options in delaying disease advancement.

SYD985 will face significant competition from other therapies approved for this treatment setting if approved, with the approvals of Tukysa, Enhertu, and Margenza making heavily pre-treated patient population a crowded space. However, these topline results are an encouraging step forward for Byondis as it seeks approval of the drug. While these Phase III results are positive and will likely result in approval in this setting, SYD985's profitable potential is restricted by the progressively crowded third-line market and company's limited oncology marketing experience and resources in comparison to competitors, particularly Daiichi Sankyo and AstraZeneca.

Even though SYD985 did show an advantage over physician's choice of therapy, it is not likely to become the new standard of care over agents with superior efficacy in this setting such as Enhertu and Tukysa. Both Enhertu and SYD985 have been linked with high rates of interstitial lung disease (ILD) and treating patients sequentially with each of these agents could lead to higher-grade adverse effects involving lung toxicity. Physicians may be wary about this when treating patients who have previously received Enhertu, which may limit SYD985's uptake.

Rinvoq for Crohn's Disease

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
Rinvoq	AbbVie Inc.	N/A	Crohn's Disease	05/26/2023	PDUFA for sNDA - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
NDA	Autoimmune/immunology	NME	86.4%	96.0%	**Average**

Rinvoq, also known as upadacitinib, is a drug developed by AbbVie for the treatment of various inflammatory diseases. The drug has already been approved for the treatment of rheumatoid arthritis, psoriatic arthritis, and atopic dermatitis. However, AbbVie is seeking approval for Rinvoq for a new indication - Crohn's Disease (CD). CD, a form of inflammatory bowel disease, is characterized by chronic inflammation of the small and large intestine. The disease can cause a range of symptoms, including abdominal pain, diarrhea, and weight loss. Currently, there are several drugs approved for the treatment of CD, but there is still a significant unmet need for new therapies that can effectively manage the condition.

AbbVie submitted a New Drug Application (NDA) for Rinvoq for the treatment of CD to the U.S. Food and Drug Administration (FDA) in July 2022. The FDA has set a Prescription Drug User Fee Act (PDUFA) date of May 26, 2023, to make a decision on the approval of the drug.

Rinvoq has shown promising results in clinical trials for the treatment of CD. In the Phase III U-EXCEL trial, Rinvoq demonstrated superiority over placebo in achieving early response, including clinical remission, endoscopic response, and CS-free clinical remission. This trial enrolled 526 participants and was a multicentre, randomized, double-blind, placebo-controlled induction study of the efficacy and safety of Rinvoq in subjects with moderately to severely active CD who have inadequately responded to or are intolerant to conventional therapies but have not failed biologic therapy. The Phase III maintenance and long-term extension study, U-ENDURE, showed similar results. Among patients with moderate to severe active CD who respond to Rinvoq induction therapy, maintenance treatment with Rinvoq was superior to placebo for all clinical and endoscopic outcomes at week 52. Already being approved for other inflammatory diseases means that the drug has been extensively studied and its safety profile is well-established. This is an advantage, as it reduces the risk of unexpected safety concerns arising during the approval process.

Currently, the market does not have any approved JAK inhibitors for CD. Galapagos' Jyseleca was set to compete against Rinvoq (upadacitinib) but given the recent Phase III DIVERSITY data failure announced in February 2023, Rinvoq is expected to launch unchallenged, as the only JAK/STAT inhibitor in CD, by mid-2023. AbbVie is positioning Rinvoq as its next-generation product since its blockbuster anti-TNF, Humira, is now facing biosimilar competition in the US.

NovaTears for Dry Eye (Ophthalmology)

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
NovaTears	Bausch Health Companies Inc.	Novaliq	Dry Eye (Ophthalmology)	06/28/2023	PDUFA for NDA - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
NDA	Ophthalmology	New Molecular Entity (NME)	73.1%	65.0%	**Above**

NovaTears, a first-in-class, preservative-free eye lubricant and tear film stabilizer that consists of perfluorohexyloctane, is under development by Bausch Health Companies and Novaliq for the treatment of evaporative dry eye diseases., NovaTears was launched in New Zealand and approved in Australia in the Fall of 2017. In January 2018, Novaliq began evaluation of NovaTears in patients in the United States with the Phase II SEECASE study. Bausch Health and Novaliq announced the submission of a New Drug Application (NDA) at the end of June 2022 to the U.S. Food and Drug Administration (FDA) seeking approval NovaTears with a proposed indication of treating the signs and symptoms of dry eye disease (DED) associated with Meibomian gland dysfunction (MGD).

The Phase II SEECASE study evaluated the effect of NovaTears at two different dosing regimes on signs and symptoms in 399 patients with a history of dry eye disease (DED). Final results of the study were encouraging, demonstrating that NovaTears improves the signs and symptoms of dry eye disease associated with meibomian gland dysfunction (MGD). NovaTears significantly improved total corneal fluorescein staining (tCFS) over control at eight weeks for both dosing regimens. Effects on tCFS started at two weeks after the start of treatment and were maintained over the study duration. Symptoms of dry eye disease were also improved with NovaTears, and this was statistically significant at week eight.

The U.S. clinical development program and NDA submission for NovaTears includes data from two Phase III clinical studies, GOBI and MOJAVE. Topline results from the pivotal Phase III GOBI trial were positive, confirming the benefit seen in the Phase II SEECASE study. The study met both of its co-primary endpoints of improved tCFS at the eight-week mark in DED with associated MGD and improved dryness score. Furthermore, the Phase III MOHAVE study also met both primary endpoints of change from baseline in tCFS at day 57 using the National Eye Institute scale and change from baseline in dryness score at day 57, rated on a visual analog scale, with statistical significance.

These positive data from GOBI and MOHAVE fulfill the FDA requirement of demonstrating safety and efficacy in two adequate and well-controlled Phase III trials. NovaTears has the potential to bring a new mechanism of action to the DED space and address a high unmet need, particularly in patients with MGD, where the drug is thought to mitigate excessive evaporation and stabilize the tear film lipid layer. The FDA has set a PDUFA date of June 28, 2023 for the application.

SRP-9001 for Duchenne Muscular Dystrophy

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
SRP-9001	Sarepta Therapeutics, Inc.	Roche	Duchenne Muscular Dystrophy (DMD)	05/29/2023	PDUFA for BLA - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
BLA	Metabolic	Biologic	87.5%	81.0%	**Above**

SRP-9001 is an investigational gene transfer therapy intended to deliver its micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein. In 2018, the company received Orphan Drug designation from the US FDA for SRP-9001 for the treatment of Duchenne muscular dystrophy (DMD). In 2020, the drug received Orphan Drug status in Europe, Fast Track status, and a Rare Pediatric Disease designation. The company has studied SRP-9001 in several studies including the Phase Ib ENDEAVOR study, a Phase I/IIa study, a Phase II study, the Phase III EMBARK study, and the planned Phase III ENVISION and ENVOL studies. In September 2022, Sarepta submitted a BLA to the US FDA for the accelerated approval of SRP-9001 to treat ambulant patients with DMD.

The BLA is based on pre-clinical, biomarker and clinical functional results. In SRP-9001's clinical trials, the drug demonstrated positive results at multiple time points, including one-, two- and four-years after treatment, in addition to a consistent safety profile. Sarepta's recently fully enrolled multinational, randomized, double-blind, placebo-controlled systemic gene delivery EMBARK study has been proposed to serve as the post-marketing confirmatory study to support the accelerated approval. The Phase II 102 study showed mixed results, with the primary biological endpoint of micro-dystrophin protein expression at week 12 differing considerably from the Phase I/IIa 101 study. In addition to this, the DMD drug failed to meet the North Star Ambulatory Assessment (NSAA) primary functional endpoint. On the other hand, there was a numerical improvement in NSAA score at 48 weeks from baseline, but this was primarily driven by patients aged 4-5 years. Those aged 6-7 years did not replicate this trend. Results from the Phase Ib ENDEAVOR study seemed to address the issue of an imbalance between placebo and SRP-9001 arms in the 6–7-year-old cohort baselines. The data also showed much larger values in micro-dystrophin expression at 12 weeks, which was statistically significant when comparing baseline and post-treatment measures.

On March 16, 2023, Sarepta announced that, at its late-cycle meeting for the SRP-9001 BLA, the US FDA determined that an advisory committee meeting will be held for SRP-9001 in advance of the PDUFA date of May 29, 2023. The outcome of this meeting will be a good indicator of whether this drug will be approved. If this drug is approved, this will be Sarepta's fourth approval in the DMD indication and will become one of six drugs approved for these patients.

Elfabrio for Fabry's Disease

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
Elfabrio	Chiesi Farmaceutici S.p.A.	Protalix BioTherapeutics, Inc.	Fabry's Disease	05/09/2023	PDUFA for BLA - Second Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
BLA	Metabolic	Biologic	87.5%	81.0%	**Above**

Elfabrio is a plant cell culture-expressed, and chemically modified stabilized version of the recombinant α-Galactosidase-A enzyme. In January 2018, the US FDA granted Fast Track designation to Elfabrio for the treatment of Fabry's disease. Elfabrio has been studied in multiple pivotal Phase III studies and is currently under review by the US FDA for the treatment of Fabry's Disease with a PDUFA date of May 9, 2023.

The company initiated the pivotal Phase III BALANCE study in June 2016, with the initial data showing mixed results. The pivotal Phase III BRIDGE study was subsequently initiated in early 2017, with the preliminary results showing Elfabrio reversing a deterioration trend in patient's kidney function. These positive results were the first out of the three Phase III studies comparing Elfabrio to two currently available therapies targeting globotriaosylceramide glycolipid for Fabry's disease: Sanofi's Fabrazyme and Takeda's Replagal. In this study, patients showed a statistically significant improvement in the estimated glomerular filtration rate after six months of treatment. In 2018, the pivotal Phase III BRIGHT study was initiated, with preliminary results released in 2019. The results demonstrated that Elfabrio was present and remained active in the plasma over the four-week infusion intervals. The positive preliminary PK data suggested that the treatment could be administered in monthly infusions compared to Fabrazyme's and Replagal's biweekly infusions. In 2020, Protalix and Chiesi submitted a BLA to the US FDA for Elfabrio in Fabry's disease (via the Accelerated Approval pathway) based on the results from multiple early-stage studies and the Phase III BRIDGE switch-over study. The companies received a Complete Response Letter in 2021 due to manufacturing facility inspection issues.

In November 2022, the companies resubmitted their BLA to the FDA with a comprehensive set of clinical and manufacturing data, including results from all three Phase III studies, BALANCE, BRIDGE, and BRIGHT. The response was accepted in December as a complete, class 2 response with a PDUFA date set for May 9, 2023. If approved, Elfabrio will be one of five approved drugs for Fabry's disease, including those approved outside of the US. With Elfabrio targeting globotriaosylceramide glycolipid, the drug will potentially compete with Fabrazyme and Replagal.

Roctavian for Hemophilia A

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
Roctavian	BioMarin Pharmaceutcal Inc.	St. Jude Children's Research Hospital UCL	Hemophilia A	06/30/2023	PDUFA for BLA - Second Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
BLA	Hematology	Biologic	92.6%	67.0%	**Above**

Roctavian (valoctocogene roxaparvovec) is an investigational AAV5 gene therapy under development by BioMarin Pharmaceutical for the treatment of severe hemophilia A. The therapy has received Orphan Drug Designation in both the U.S. and Europe. In August 2022 Roctavian was granted conditional marketing authorization by the European Commission for the treatment of severe hemophilia A (congenital Factor VIII deficiency) in adult patients without a history of Factor VIII inhibitors and without detectable antibodies to adeno-associated virus serotype 5 (AAV5).

BioMarin initially filed for approval with the FDA in December 2019, but was issued a Complete Response Letter in August 2020 requesting that the company complete its pivotal Phase III GENEr8-1 study and submit two-year follow-up safety and efficacy data on all study participants. The FDA's rejection of BioMarin's BLA seems to have been based on differences between Phase I/II and Phase III trials, which prevented the agency from using the earlier trial to evaluate the treatment's durability of effect.

Five year follow -up data from a Phase I/II POC study of Roctavian in 15 patients were largely positive, demonstrating impressive safety and durable. In year 5, the annualized bleed rate (ABR) in the high dose cohort remained stable at 0.7 bleeds per year, in line with what is achieved by market leader Hemlibra or replacement factor VIII (rfVIII) therapies (ABR = >1.5). Additionally, the use of fVIII infusions declined by 96% through five years compared to baseline. Importantly, these results continue to suggest patients will be free from infusions for eight years or more.

The Phase III GENEr8-1 study represents the largest gene therapy trial in the hemophilia space, with 112 rollover patients completing a baseline observational study prior to infusion. A single infusion of Roctavian reduced the ABR in these patients by 84% ($p<0.0001$) from 4.8 to 0.8 bleeds per year, demonstrating that the gene therapy was comparable to prophylactic fVIII, with the substantial advantage of a long dosing interval. Moreover, the reliance on bi-weekly fVIII infusion was reduced by 99%. The treatment was fast-acting, with 67% of patients becoming fVIII infusion-free within five weeks, and 79.5% becoming bleed-free versus 32.1% at baseline.

BioMarin resubmitted its BLA in September 2022, incorporating two-year outcomes from the global GENEr8-1 Phase III study and supportive data from five years of follow-up from the ongoing Phase I/II dose escalation study. The FDA has set a PDUFA date of June 30, 2023 for the application.

TransCon PTH for Hypoparathyroidism

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
TransCon PTH	Ascendis Pharma A/S	Visen	Hypoparathyroidism	04/30/2023	PDUFA for NDA - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
NDA	Endocrine	Non-NME	93.7%	64.0%	**Above**

Ascendis Pharma is currently developing TransCon PTH, palopegteriparatide, an extended-release self-injectable prodrug of parathyroid hormone (PTH), for the treatment of hypoparathyroidism aiming to elevate thyroid levels back into an expected range. The transient conjugation of PTH facilitates the release of the PTH for an extended duration, to improve the efficacy, safety, and dosing frequency of the drug. This development has been awarded an Orphan Drug Designation in the United States, due to hypoparathyroidism being considered a rare disease.

In March 2022, Ascendis released data from the Phase III PaTHway study which showed that 78.7% of palopegteriparatide-treated patients achieved serum calcium levels in the normal range compared to 4.8% for patients in control group (p-value = <0.0001). Data showed a statistically significant reduction in patient-reported disease impact, and patient-reported disease-specific physical and cognitive symptoms whilst showing improvements in patient-reported physical functioning compared to patients in control group. The US-based study also showed that at Week 26, 95% of TransCon PTH-treated patients were able to discontinue conventional treatments with therapeutic levels of calcium supplements and active vitamin D. Looking at data from the PaTHway study in Japan, this study replicated the positive outcomes, where twelve out of thirteen patients met the primary composite endpoint which was defined as serum calcium levels in the normal range (8.3–10.6 mg/dL) and independence from conventional therapy.

A New Drug Application (NDA) for palopegteriparatide in adult patients with hypoparathyroidism was submitted in August 2022, and the U.S. Food & Drug Administration (FDA) accepted this application for priority review in October 2022. The Prescription Drug User Fee Act (PDUFA) target action date of April 30, 2023, has been set and if approved, will be the first approval in the hypothyroidism market since 2015.

Momelotinib for Myelofibrosis

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
Momelotinib	GSK plc	Gilead Sciences	Myelofibrosis (MF)	06/16/2023	PDUFA for NDA - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
NDA	Neurology	NME	87.7%	64.0%	**Above**

On June 16, 2023, the United States Food and Drug Administration (FDA) is set to make a decision regarding the approval of GSK's Momelotinib for the treatment of myelofibrosis with anemia. Myelofibrosis, a rare form of blood cancer which occurs when the bone marrow produces abnormal blood cells, can lead to anemia, fatigue, and an enlarged spleen. GSK's Momelotinib is an oral JAK1/JAK2/ACVR1 inhibitor that is intended to reduce spleen size, improve anemia, and alleviate symptoms associated with myelofibrosis. Evidence suggests that JAK1/2 inhibition is responsible for improving splenomegaly, and AVCR1 inhibition reduces circulating hepcidin, a hormone that is often elevated in myelofibrosis and contributes to anemia.

GSK's Momelotinib has been in Phase III clinical development since November 2019 and has to date been evaluated in three Phase III trials. The New Drug Application for Momelotinib, which was accepted by the FDA in August 2022, was based on the results from key Phase III trials, including the pivotal MOMENTUM trial, which met all its primary and key secondary endpoints. Furthermore, in the Phase III Simplify 1 trial, Momelotinib demonstrated a significant reduction in spleen size, improvement in anemia, and a better safety profile compared to the current standard of care, JAK1/2 inhibitor ruxolitinib (Jakafi). This suggests that Momelotinib may offer a better treatment option for the disease. Regarding other treatment options, Fedratinib, a JAK2/FLT3 inhibitor, is US FDA approved but, as with ruxolitinib, is hindered by exacerbating anemia. Consequently, there is an unmet need for agents that can ameliorate anemia in myelofibrosis, wherein GSK hopes Momelotinib will have its success.

However, there are some potential roadblocks to the approval of Momelotinib. One concern is the potential for long-term side effects, as JAK inhibitors have been associated with an increased risk of infections and other serious adverse events. Despite this, if approved, Momelotinib could provide a much-needed alternative treatment option for patients with myelofibrosis with anemia.

Ocaliva for Non-Alcoholic Steatohepatitis (NASH)

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
Ocaliva	Intercept Pharmaceuticals, Inc.	ADVANZ PHARMA	Non-Alcoholic Steatohepatitis (NASH)	06/22/2023	PDUFA for NDA - Second Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
NDA	Endocrine	New Molecular Entity (NME)	78.7%	64.0%	**Below**

Obeticholic acid (OCA; Ocaliva) is a potent and selective farnesoid X receptor (FXR) agonist under development by Intercept Pharmaceuticals for the treatment of patients with pre-cirrhotic liver fibrosis due to non-alcoholic steatohepatitis (NASH). It is a derivative of natural human bile acid CDCA (chenodeoxycholic acid) and was granted a Breakthrough designation by the US FDA in January 2015. In June 2023, the US FDA is anticipated to make an approval decision following a class 2 resubmission of obeticholic acid's NDA in December 2022.

This is not the first regulatory submission for obeticholic acid in this indication, as it initially received a complete response letter (CRL) from the FDA in June 2020, given the agency was uncertain on whether the clinical benefits outweighing the risks. In December 2022, Intercept Pharmaceuticals resubmitted the NDA in response to the CRL. The NDA resubmission included more robust data from the pivotal Phase III REGENERATE study showing that treatment with obeticholic acid 25 mg demonstrated a statistically significant greater increase in the proportion of recipient achieving an improvement in liver fibrosis by at least 1 stage without worsening of NASH versus placebo; this improvement was more pronounced in individuals with more advanced disease at baseline. However, in the Phase III REVERSE study, the primary endpoint of ≥ 1-stage histological improvements in fibrosis with no worsening of NASH in compensated cirrhosis patients after up to 18 months of therapy was not met. This will limit the size of the potentially eligible patient population.

Intercept Pharmaceuticals took approximately 18 months to submit a response to the complete response letter, which was accepted by the FDA in January 2023. During that interval, Intercept supplied additional data, as explained in FDA meetings, though their MAA which was withdrawn has yet to be refiled. The second review for the PDUFA date is set for June 22, 2023. If approved, this would be the first drug on the US market for the treatment of patients with pre-cirrhotic liver fibrosis due to non-alcoholic steatohepatitis.

IPX203 for Parkinson's Disease (PD)

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
IPX203	Amneal	N/A	Parkinson's disease (PD)	06/30/2023	PDUFA for NDA - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
NDA	Neurology	New Molecular Entity (NME)	87.3%	64.0%	**Above**

Amneal pharmaceuticals is currently developing IPX203, an innovative extended-release form of carbidopa/levodopa (CD/LD), for the treatment of Parkinson's disease (PD). The company announced a New Drug Application (NDA) was accepted for review by the U.S. Food and Drug Administration (FDA) in November 2022. This NDA was based on data from the Phase III RISE-PD clinical trial, where treatment with IPX203 provided considerably more "Good On" time and less "Off" time compared to immediate-release CD/LD, despite being dosed less frequently. Overall, a greater proportion of IPX203 patients experienced improvement compared with immediate release CD/LD. The MDS-UPDRS scores were similar between the two treatments. In addition, a post-hoc analysis at 20 weeks showed that IPX203 extended its "appropriate duration" by 1.55 hours with a single dose compared with immediate-release CD/LD. The FDA has set a Prescription Drug User Fee Act (PDUFA) deadline of June 30, 2023, to complete their assessment of the NDA.

Amneal's marketing approach for IPX203 will emphasize its clinical efficacy over immediate-release CD/LD, which provides more consistent symptom coverage throughout the day, benefiting patients who experience fluctuations. While the convenience of IPX203's less frequent dosing compared to Rytary is an advantage, it may not compete well against cheaper Rytary generics, particularly for patients responding well to Rytary. The success of IPX203's market launch will depend on addressing market access and insurance issues, as well as educating providers on the conversion process.

ABBV-951 for Parkinson’s Disease

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
ABBV-951	AbbVie Inc.	N/A	Parkinson's Disease (PD)	05/20/2023	PDUFA for Approval - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
NDA	Neurology	Non-NME	82.8%	64.0%	**Above**

AbbVie's ABBV-951 (foscarbidopa/foslevodopa) is currently awaiting approval by the US Food and Drug Administration (FDA) for the treatment of motor fluctuations in patients with advanced Parkinson's disease. ABBV-951 is a subcutaneously delivered combination solution of carbidopa and levodopa (CD/LD) prodrugs, both of which are already widely used in the treatment of Parkinson's disease. ABBV-951 aims to deliver a more consistent and continuous supply of CD/LD to the brain, which may help to reduce motor fluctuations and improve the overall quality of life for patients with advanced Parkinson's disease whose motor symptoms are not controlled by oral medications.

ABBV-951’s New Drug Application (NDA) was submitted in May 2022 and was based on results from the Phase III M15-736, head-to-head, randomized and controlled clinical trial, which demonstrated statistically significant improvement in "On" time without troublesome dyskinesia compared to oral immediate-release CD/LD. The NDA is also supported by data from the 52-week, Phase III M15-741 open-label study. Data has shown ABBV-951’s safety and tolerability are comparable to oral CD/LD, with the incidence of serious adverse events being 7% and 6% in the ABBV-951 group and oral CD/LD group, respectively.

If approved, ABBV-951 would provide a new treatment option for patients with advanced Parkinson's disease, a population that currently has limited treatment options. It would also represent an important advance in the treatment of motor fluctuations, which are a significant source of disability and reduced quality of life for patients with Parkinson's disease. ABBV-951 is likely to face competition from other pipeline drugs, many of which are in Phase III clinical development, although it could be first to reach the market.

Abrysvo for Respiratory Syncytial Virus (RSV) Prevention

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
Abrysvo	Pfizer	N/A	Respiratory Syncytial Virus (RSV) Prevention	05/01/23 - 05/31/23	PDUFA for BLA - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
BLA	Respiratory	Biologic	N/A	93%	Above

Abrysvo is Pfizer's respiratory syncytial virus (RSV) vaccine candidate based on a prefusion form of the RSV F protein.

On December 2022, Pfizer announced that it had submitted a Biologics License Application (BLA) for its RSV vaccine candidate, PF-06928316 or RSVpreF, to the U.S. Food and Drug Administration (FDA) for priority review for the prevention of lower respiratory tract disease caused by RSV in individuals 60 years of age and older. The Prescription Drug User Fee Act (PDUFA) goal date for a decision by the FDA on the RSVpreF application was set for May 2023. This decision followed the FDA's granting of Breakthrough Therapy Designation to RSVpreF in older adults in March 2022.

The regulatory submission was supported by results from the Phase III clinical trial RENOIR (RSV vaccine Efficacy study iN Older adults Immunized against RSV disease). RENOIR was a global, randomized, double-blind, placebo-controlled study designed to assess the efficacy, immunogenicity, and safety of a single dose of RSVpreF in adults 60 years of age and older. RENOIR enrolled approximately 37,000 participants, randomized to receive RSVpreF 120 μg or placebo in a 1:1 ratio. In August 2022, Pfizer announced positive top-line results from RENOIR with vaccine efficacy of 85.7% reported for subjects with lower respiratory tract illness (LRTI) defined by analysis of three or more RSV-associated symptoms.

During the advisory panel meeting held on February 2023, panellists voted 7-4 with one abstention that the available data were adequate to support the safety and effectiveness of Pfizer's Abrysvo (RSVPreF) when administered to individuals 60 years of age and older for the prevention of lower respiratory tract disease caused by RSV. However, only three committee members endorsed both the vaccine's safety and efficacy profile. There was no separate vote on whether the benefits of the vaccine outweighed the risks, and advisers who voted against encouraged the FDA to wait until Phase III trials are complete with Pfizer before making an approval decision.

Arexvy for Respiratory Syncytial Virus (RSV) Prevention

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
Arexvy	GSK plc	Agenus Inc.	Respiratory Syncytial Virus (RSV) Prevention	05/03/2023	PDUFA for BLA - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
BLA	Infectious Diseases	Biologic	80.0%	85.0%	**Above**

GSK's Arexvy, an adjuvanted, recombinant protein vaccine candidate for respiratory syncytial virus (RSV) prophylaxis in the older adult population, has an upcoming US FDA approval decision on May 3, 2023. It is set in place as the frontrunner position in the race to steal first-to-market status in elderly RSV prophylaxis. RSV is a highly contagious virus that causes respiratory infections in people of all ages. However, it is most severe in infants, young children, and older adults, and can lead to hospitalization, especially in those with weakened immune systems. By preventing contraction of RSV, Arexvy will not only save lives, but also reduce complications, costs, and burdens for healthcare practitioners and patients.

Latest data from the Phase III AReSVi 006 trial involving 24966 participants were published in February 2023 in the New England Journal of Medicine. The promising results showed that a single dose of the RSVPreF3 OA vaccine had an acceptable safety profile and prevented RSV-related acute respiratory infection and lower respiratory tract disease (LRTD) and severe RSV-related LRTD in adults 60 years of age or older, regardless of RSV subtype and the presence of underlying coexisting conditions. Over a median follow-up of 6.7 months, vaccine efficacy against reverse transcriptase polymerase chain reaction (RT-PCR)–confirmed RSV-related LRTD was 82.6%, with seven cases (1.0 per 1000 participant-years) in the vaccine group and 40 cases (5.8 per 1000 participant-years) in the placebo group. Vaccine efficacy was 94.1% (95% CI, 62.4 to 99.9) against severe RSV-related LRTD (assessed on the basis of clinical signs or by the investigator) and 71.7% (95% CI, 56.2 to 82.3) against RSV-related acute respiratory infection. Vaccine efficacy was similar against the RSV A and B subtypes (for RSV-related LRTD: 84.6% and 80.9%, respectively; for RSV-related acute respiratory infection: 71.9% and 70.6%, respectively). High vaccine efficacy was observed in various age groups and in participants with coexisting conditions.

On March 1, 2023, the FDA's Vaccines and Related Biological Products Advisory Committee (VRBPAC) met to discuss and make recommendations on the safety and effectiveness of GSK's Arexvy with a requested indication for active immunization for the prevention of LRTD caused by respiratory syncytial virus RSV-A and RSV-B subtypes in adults 60 years of age and older. The Committee voted 10-2 that the available data are adequate to support the safety of GSK's adjuvanted vaccine Arexvy (RSVPreF3+ASO1E) when administered to individuals 60 years of age and older for the prevention of LRTD caused by RSV, while it voted unanimously that the data are adequate to support the vaccine's effectiveness.

Overall, with continued positive data releases and the FDA committee decision in favour of Arexvy, GSK remains on track to be the first approved RSV vaccine for older adults come May 3, 2023.

Aripiprazole 2-Month for Schizophrenia

The development of a 2-month long-acting injectable (LAI) formulation of aripiprazole is currently being undertaken by

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
Aripiprazole 2M	H. Lundbeck A/S	Otsuka	Schizophrenia	04/27/2023	PDUFA for BLA - First Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
NDA	Psychiatry	Non-NME	99.9%	64.0%	**Average**

Lundbeck and Otsuka. Although aripiprazole is classed as an atypical antipsychotic, it is different from other atypical agents due to it being a partial agonist at the dopamine D2 receptor, rather than an antagonist, and it does not have a lower affinity there than at the serotonin 5-HT2A receptor. By blocking overstimulated receptors and stimulating underactive ones, partial D2 agonists act as dopamine stabilizers.

Filing for approval in Canada was submitted in January 2023. The U.S. Food and Drug Administration (FDA) accepted a New Drug Application (NDA) for the drug in September 2022. Regulatory approval would be based on a multiple-dose, randomized, parallel-arm, clinical trial which assessed the safety, tolerability, and pharmacokinetics of 2-month aripiprazole in adults with schizophrenia or bipolar I disorder. Otsuka Pharmaceutical Europe and Lundbeck also announced that the European Medicines Agency (EMA) accepted the Marketing Authorisation Application (MAA) for aripiprazole 2-month for the maintenance treatment of schizophrenia in adult patients stabilised with aripiprazole in June 2022.

Findings from the pivotal Phase I study that enrolled 266 participants revealed that aripiprazole 2-month met the primary endpoint criteria by establishing similar aripiprazole plasma concentrations, and thus comparable effectiveness, to Otsuka and Lundbeck's aripiprazole once-monthly (Abilify Maintena), over a two-month dosing interval.

The launch of aripiprazole 2-month is planned to coincide with Abilify Maintena's loss of market exclusivity, which the companies hope will limit genericization. The crowded LAI schizophrenia market is currently led by Johnson & Johnson's Invega franchise. If approved in the US, aripiprazole 2-month's biggest competition will be from Alkermes's Aristada, an already marketed aripiprazole formulation administered once every two months.

Botulax for Wrinkles

Drug	Company	Partner(s)	Indication(s)	Date Range	Expected Catalyst(s)
Botulax	Hugel Pharma Co, Ltd	CROMA Pharma	Wrinkles	04/06/2023	PDUFA for BLA - Second Review
Phase	**Disease Group**	**Drug Class**	**Group/Class PoS**	**Group/Class LOA**	**BMT LOA Opinion**
BLA	Dermatology	Biologic	99.9%	64.0%	**Average**

Letybo (letibotulinumtoxinA) is a protein separated from clostridium botulinum type A. It is a toxin protein purified from type A botulism which paralyzes muscles by blocking secretion of the neurotransmitter acetylcholine. Known as Botulax in Korea, Letybo's targets include synaptobrevin, syntaxin and SNAP-25. Currently under development by Hugel Pharma in partnership with CROMA Pharma, Letybo has been a market leader in Asia.

The filing for approval of Letybo is supported by the data from the pivotal Phase III BLESS trial which demonstrated high efficacy and a convincing safety profile in the treatment of glabellar lines (GL). The results from the Phase III BLESS trial showed that at 4 weeks, 78.6% of the active treatment subjects were responders based on the investigator's assessment and 68.8% based on the subject's assessment, resulting in a composite responder rate of 64.7% for the active treatment group, whereas the corresponding rate was 0.0% in the placebo group ($P < 0.001$). Subjects noted a substantial improvement in GL severity as early as day 2, with the median time to onset of effect being 3 days. The mean time until first retreatment for the Letybo group was 127 days.

In an increasingly crowded botulinum toxin aesthetics market in the US, where AbbVie's first-to-market Botox remains the traditional best seller, Letybo is unlikely to take a significant portion of the market. The positive opinion of approval in Europe was based on the collection of 3 Phase III BLESS studies, which in total enrolled over 1,000 participants. It is important to note that, as of yet, the results from a comparative US trial between Letybo and Botox have not been released – and these results could be influential in distinguishing Letybo from competitors and result in a shift in the current market.

Devices

Omnipod 5 for Diabetes Mellitus, Type II

Device	Company	Partner(s)	Market(s)	Date Range	Expected Catalyst(s)
Omnipod 5	Insulet Corporation	DexCom	Diabetes Mellitus, Type II	Now – 06/30/2023	510(k) Approval Decision

Insulet submitted a 510(k) application for the Omnipod 5, a basal-only Pod targeted for those with type 2 diabetes, on November 30, 2022. The device delivers a constant rate of rapid-acting insulin for 72 hours without the necessity of a PDM/Controller or a phone app. The application was based on encouraging findings from a 21-week research involving 29 type 2 diabetes individuals.

The study demonstrated a significant reduction in HbA1c levels by 1.3% (14.2 mmol/mol) and an improvement in Time in Range (TIR) by 4.6 hours per day during the eight-week study compared with baseline. Additionally, hypoglycemia was reduced by 4 minutes per day in the group previously using multiple daily injections and did not change for the group previously using basal-only injections. The improvements in glycemic control were achieved alongside a reduction in insulin use (-29 units per day, or 31.4%) for the prior multiple daily injections group (no change for prior basal-only injection group) and with no change in Body Mass Index (BMI) in either group. Participants in the study were aged (mean±SD) 61±8y with BMI 33.9±4.4kg/m2, diabetes duration 19±9y, and baseline A1C 9.4±0.9% (range: 8.1-11.7%). Mean A1C decreased to 8.0±0.7% after 8 weeks of AID ($p<0.05$). After an additional 13 weeks of use, mean A1C was 7.7±0.7%, corresponding to an overall decrease of 1.6±1.0% from baseline to 21 weeks of total use ($p<0.05$).

Fourteen participants who volunteered for a post-study human factors interview reported a System Usability Scale of 90.5 after the conclusion of the study. The submission of the 510(k) application for the basal-only Omnipod 5 suggests that Insulet is taking steps to improve the lives of type 2 diabetes patients. This device could provide a simpler and more effective treatment option for individuals who require basal insulin delivery. If approved by the FDA, the Omnipod 5 could be a game-changer for those with type 2 diabetes.

Based on the FDA's guidelines for 510(k) approvals, we estimate an approval decision for this device for this indication will be granted in approximately in April 2023.

Deals

Adaptimmune Acquisition of TCR² Completed

Expected Date Range – 03/06/2023 – 06/30/2023

On March 6, 2023, Adaptimmune announced the company entered into an agreement to which the company will combine with TCR² Therapeutics in all-stock transaction. Adaptimmune shareholders will own approximately 75% and TCR² stockholders will own approximately 25% of the combined company when the transaction closes. The combined company will trade on the Nasdaq Stock Market under the symbol “ADAP” with an expected cash runway extended into 2026. The closing of the transaction is expected in the second quarter of 2023.

The combination will bring forth a distinguished cell therapy company with a clinical stage pipeline focused on solid tumors and years of experience in T-cell therapy manufacturing. Throughout 2023, the combined company plans to advance its combined pipeline including afami-cel, ADPA2M4CD8, gavo-cel and TC-510. The rolling BLA submission of afami-cel for synovial sarcoma, supported with encouraging results from the pivotal Phase II SPEARHEAD-1 study, is expected to be completed by mid-2023. Alongside, monotherapy data is expected from the Phase I SURPASS trial of ADP-A2M4CD8 in solid tumors as well as data readout from the Phase II trial of gavo-cell in platinum resistant or refractory ovarian cancer. TCR²’s next generation product, TC-510, in solid tumors has a Phase I data readout planned for the end of 2023. The pipeline also extends to various development catalysts and preclinical programs planned through 2024.

Amgen Acquisition of Horizon Completed

Expected Date Range: Now – 06/30/2023

On December 12, 2022, Amgen announced plans to buy Dublin-based Horizon Therapeutics for $116.50 per share in cash (a 51% premium to Horizon’s 10-day average price of $76.97 before the talks became public on November 29). The acquisition values the entire issued and to be issued ordinary share capital of Horizon at about $27.8bn on a fully diluted basis and implies an enterprise value of around $28.3bn. The acquisition is expected to close in the first half of 2023.

Founded in 2008, Horizon is focused on treatments for rare autoimmune and severe inflammatory diseases. Its best-seller, Tepezza (teprotumumab), received FDA approval in early 2020 as the first treatment for thyroid eye disease. The orphan drug's sales reached nearly $2bn in 2022, which is more than half of the company's total sales of $3.6m. Horizon has another blockbuster product, Krystexxa (pegloticase), for uncontrolled gout. In 2022, Krystexxa sales grew 37% to $716.2m. Another key product for Horizon is Uplizna (inebilizumab) for adults with neuromyelitis optica spectrum disorder. According to industry analysts, Amgen is about to lose $30bn in sales due to biosimilar drugs competition for its existing products. Amgen says the acquisition will provide it the following benefits: strengthen its portfolio of first-in-class and best-in-class therapeutics by adding a complementary portfolio of medicines from Horizon that address the needs of patients suffering from rare diseases; capitalize on its 20-year commercial and medical legacy in inflammation and nephrology and its global scale to enhance the growth potential of Horizon’s portfolio; utilize Amgen’s industry-leading R&D, process development and global manufacturing expertise in biologic medicines for the benefit of Horizon’s approved medicines and potential new medicines; and generate robust cash flow (approximately $10bn combined over twelve months through Q3 2022) to support capital allocation priorities, including ongoing investment in innovation and continued dividend growth. The companies expect to save about $500m annually in costs by year three of the merger.

Chiesi Farmaceutici Acquisition of Amryt Pharma Completed

Expected Date Range: Now - 06/30/2023

On January 8, 2023, privately held Italian biotech Chiesi Farmaceutici S.p.A. entered into a definitive agreement to acquire Dublin-headquartered Amryt Pharma plc (AMYT) for up to $1.48bn in cash. Chiesi currently earns most of its revenues in respiratory medicine in Europe, but this deal enables it to broaden its focus into rare disease and expand its US market presence.

Amryt was established in 2015 after the acquisition of Birken AG (skin care products and medicines developed from natural substances including betulin, a topical gel derived from birch bark). Nasdaq-listed Amryt has a portfolio of rare and orphan disease therapeutics. Its current top-selling drug is Myalept/Myalepta (metreleptin), approved in both the US and EU to treat complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy. Amyrt's two other commercialized products are Mycapssa (octreotide), an oral somatostatin analog for the growth hormone disorder acromegaly, and Juxtapid/Loxujta (lomitapide), an adjunct to a low-fat diet and other lipid-lowering medicines for adults with homozygous familial hypercholesterolemia, a rare cholesterol disorder. Chiesi aims to expand revenues for the overall Amryt business, which was forecast to reach between $260-270m for 2022.

Amryt's pipeline is led by Oleogel-S10 (Filzuvez), a betulin-rich birch bark extract. The therapy was approved in Europe in 2016 for wound healing and in June 2022 for epidermolysis bullosa (EB), but is awaiting FDA approval in the US for the EB indication, for which it has achieved Orphan Drug designation (in the US and Europe) as well as Fast Track status. Despite a June 2021 NDA filing acceptance, in February 2022, the company received a complete response letter from the US FDA requesting further evidence of Filzuvez's efficacy, rejecting the data from Amryt's Phase III EASE study. The company is expected to submit a formal dispute resolution request. In addition to its approved acromegaly indication, Mycapssa is also under investigation in neuroendocrine tumor patients with carcinoid symptoms; a Phase III study is expected to begin in H1 2023. AP103 is a preclinical gene therapy candidate expected to enter the clinic in 2023 for dystrophic EB.

Under the terms of the cash transaction, Chiesi will buy all outstanding shares of Amryt for a purchase price of $14.50 per American depositary share (a 101% premium based on Amryt ADS' 10-day volume-weighted average price), each representing five Amryt ordinary shares (or a price of $2.90 per ordinary share). Amryt is also eligible to receive contingent value rights (CVRs) of up to $2.50 per ADS ($0.50 per share) payable if certain milestones related to Filsuvez are achieved before December 31, 2024. These consist of $1.00 per ADS ($0.20 per share) upon FDA approval and $1.50 per ADS ($0.30 per share) upon successful receipt of a priority review voucher from the FDA. The total transaction value implied at close is approximately $1.25bn in up-front consideration, plus an additional potential.

Biomedtracker Pharma Intelligence

Q2 2023 Large Impact Catalysts

Meddevicetracker Pharma Intelligence

Company	Symbol	Product	Indication	Phase	Catalyst Title	Expected End Date	Catalyst
4SC AG	VSC	Resminostat	Cutaneous T-Cell Lymphoma (CTCL) - NHL	II	Phase II RESMAIN - Top-Line Results	06/22/2023	114675
AADi Bioscience, Inc.	AADI	Fyarro	Solid Tumors	Approved	Phase II PRECISION 1 - Topline Results	06/30/2023	176215
Aardvark Therapeutics, Inc.		ARD-101	Obesity	II	Phase II UCSD - Top-Line Results	06/30/2023	174645
AbbVie Inc.	ABBV	Imbruvica	Marginal Zone Lymphoma - NHL	Approved	Phase III SELENE - Top-Line Results	05/31/2023	166982
AbbVie Inc.	ABBV	Rinvoq	Crohn's Disease	NDA	PDUFA for sNDA - First Review	05/26/2023	178996
AbbVie Inc.	ABBV	Rinvoq	Crohn's Disease	NDA	European Approval Decision	05/01/2023	179001
AbbVie Inc.	ABBV	GLPG2737	Polycystic Kidney Disease	Development Outside U.S.	Phase II MANGROVE - Top-Line Results	06/30/2023	173184
Achieve Life Sciences, Inc.	ACHV	Cytisine	Smoking Cessation	III	Phase III ORCA-3 - Topline Results	06/30/2023	180621
Acticor Biotech, SAS		ACT-017	Ischemic Stroke	II/III	Type C Meeting with FDA	04/30/2023	183363
Active Implants LLC		NUsurface Meniscus Implant	Cartilage and Joint Repair	IDE	FDA Advisory Panel Brief	04/18/2023	185522
Active Implants LLC		NUsurface Meniscus Implant	Cartilage and Joint Repair	IDE	FDA Advisory Panel Meeting	04/20/2023	185523
Acutus Medical, Inc.	AFIB	AcQBlate FORCE Sensing Ablation Catheter	Atrial Fibrillation/Flutter	PMA	PMA Approval Decision	04/30/2023	181362
Adaptimmune Therapeutics plc	ADAP	GSK3377794	Sarcoma	II	Phase II IGNYTE-ESO - Top-Line Results	06/30/2023	179010
Adaptimmune Therapeutics plc	ADAP	ADP-A2M4CD8	Esophageal Cancer	II	Phase II SURPASS-2 - Top-Line Results	06/30/2023	166474
Advicenne	ADVIC	Sibnayal	Renal Disease / Renal Failure	III	Phase III ARENA-2 - Top-Line Results	06/30/2023	152738
AiCuris Anti-infective Cures GmbH		Pritelivir (Oral)	Herpes Simplex Virus (HSV) (Antiviral)	III	Phase III Dual-Resistance - Top-Line Results	05/31/2023	166486
AiCuris Anti-infective Cures GmbH		AIC649	COVID-19 Treatment	Preclinical	Phase II - Top-Line Results	06/30/2023	166489
Akari Therapeutics, Plc	AKTX	Coversin	Transplant-Associated Thrombotic Microangiopathy (TA-TMA)	III	Phase III - Pediatric HSCT-TMA - Part A Data	06/30/2023	179124
Akebia Therapeutics, Inc.	AKBA	Vafseo	Anemia Due to Chronic Kidney Disease, Dialysis-Dependent	III	Approval Decision (Europe)	05/01/2023	172539
Akero Therapeutics, Inc.	AKRO	Efruxifermin	Non-Alcoholic Steatohepatitis (NASH)	IIb	Phase IIb SYMMETRY - 12 Week Expansion Cohort Results	06/30/2023	180261
Akeso Inc.		AK101	Psoriasis	Development Outside U.S.	Phase III Monotherapy (China) - Top-Line Results	06/30/2023	174824
Akeso Inc.		AK102	Dyslipidemia / Hypercholesterolemia	Development Outside U.S.	Phase III Registrational Study (China) - Top-Line Results	06/30/2023	174825
Akston Biosciences Corporation		AKS-452	COVID-19 Prevention	Development Outside U.S.	Phase II (India) - Top-Line Results	06/30/2023	173159
Aldeyra Therapeutics, Inc.	ALDX	ADX-629	Chronic Cough	II	Phase II - Top-Line Results	06/30/2023	183097
Aldeyra Therapeutics, Inc.	ALDX	ADX-2191	Retinitis Pigmentosa (RP) (Ophthalmology)	II	Phase II Rhodopsin Mutations - Top-Line Results	06/30/2023	175538
Aldeyra Therapeutics, Inc.	ALDX	ADX-2191	Primary Central Nervous System Lymphoma (PCNSL) - NHL	NDA	PDUFA for NDA - First Review	06/21/2023	183310
Alkeus Pharmaceuticals, Inc.		ALK-001	Stargardt Disease (Ophthalmology)	II	Phase II - Top-Line Results	06/30/2023	170485
Alnylam Pharmaceuticals Inc.	ALNY	Amvuttra	Hereditary Transthyretin (hATTR) Amyloidosis With Polyneuropathy (Familial Amyloid Polyneuropathy)	Approved	Japanese Approval Decision	04/30/2023	174832
Alphamab Oncology	9966	KN046	Non-Small Cell Lung Cancer (NSCLC)	Development Outside U.S.	Phase III ENREACH-LUNG-01 - Top-Line Results	05/31/2023	162950
Alvotech	ALVO	AVT-02	Rheumatoid Arthritis (RA)	BLA	BsUFA Approval	04/13/2023	183340
Alvotech	ALVO	AVT-02	Psoriasis	BLA	BsUFA Approval	04/13/2023	183342
Alzamend Neuro, Inc.	ALZN	AL001 (Alzamend)	Alzheimer's Disease (AD)	II	Phase IIa MAD - Top-Line Results	06/30/2023	177093
Amarin Corporation plc	AMRN	Vascepa	COVID-19 Treatment	Investigator Initiated	Phase IV - MITIGATE - Top-Line Results	04/30/2023	167557
Amgen, Inc.	AMGN	Tezspire	Urticaria	II	Phase IIb INCEPTION – Top-line Results	06/30/2023	179445
Amgen, Inc.	AMGN	Tezspire	Nasal Polyposis	III	Phase III WAYPOINT - Top Line Results	05/31/2023	170795
Amgen, Inc.	AMGN	Tezspire	Chronic Rhinosinusitis	III	Phase III WAYPOINT - Top Line Results	05/31/2023	175709
Amgen, Inc.	AMGN	Tezspire	Chronic Obstructive Pulmonary Disease (COPD)	II	Phase IIa COURSE - Top-Line Results	05/31/2023	158256

Biomedtracker Pharma Intelligence

Q2 2023 Large Impact Catalysts

Meddevicetracker Pharma Intelligence

Company	Symbol	Product	Indication	Phase	Catalyst Title	Expected End Date	Catalyst
Amgen, Inc.	AMGN	Amjevita	Psoriasis	Approved	Phase III Switch Study - Top-Line Results	06/30/2023	184710
Amgen, Inc.	AMGN	Biosimilar Eculizumab (Amgen)	Paroxysmal Nocturnal Hemoglobinuria (PNH)	III	Approval Decision (Europe)	05/01/2023	185305
Amgen, Inc.	AMGN	Biosimilar Eculizumab (Amgen)	Paroxysmal Nocturnal Hemoglobinuria (PNH)	III	Phase III vs. Eculizumab (EU) - Top-Line Results	05/31/2023	176827
Amgen, Inc.	AMGN	Lumakras	Non-Small Cell Lung Cancer (NSCLC)	Approved	Phase II Lung-MAP - Top-Line Results	05/31/2023	168850
Amgen, Inc.	AMGN	ABP 654	Psoriasis	III	Phase III Switch Study - Top-Line Results	06/30/2023	184709
Amgen, Inc.	AMGN	ABP 938	Wet Age-Related Macular Degeneration (Wet AMD) (Ophthalmology)	III	Phase III vs. Eylea - Topline Results	06/30/2023	176372
Amicus Therapeutics, Inc.	FOLD	AT-GAA	Pompe Disease	BLA	PDUFA for BLA - First Review	06/30/2023	171036
Amicus Therapeutics, Inc.	FOLD	AT-GAA	Pompe Disease	BLA	PDUFA for sNDA - First Review	06/30/2023	171791
Amicus Therapeutics, Inc.	FOLD	AT-GAA	Pompe Disease	BLA	CHMP Opinion - Miglustat	06/30/2023	183856
Amneal Pharmaceuticals, Inc.	AMRX	IPX203	Parkinson's Disease (PD)	NDA	PDUFA for Approval - First Review	06/30/2023	182263
Amneal Pharmaceuticals, Inc.	AMRX	Dihydroergotamine Autoinjector	Migraine and Other Headaches	NDA	Approval Decision (US)	04/30/2023	169957
Amylyx Pharmaceuticals, Inc.	AMLX	Relyvrio	Amyotrophic Lateral Sclerosis (ALS)	Approved	CHMP Opinion	04/30/2023	173986
Amylyx Pharmaceuticals, Inc.	AMLX	Relyvrio	Amyotrophic Lateral Sclerosis (ALS)	Approved	European Approval Decision	06/30/2023	173987
Anavex Life Sciences Corp.	AVXL	ANAVEX 2-73	Parkinson's Disease Dementia (PDD)	II	Phase II OLE - Top-Line Results	06/30/2023	184238
Anji Pharmaceuticals, Inc.		ANJ908	Chronic Idiopathic Constipation	II	Phase II - POC (US/China) - Top-Line Results	05/31/2023	177470
Annexon, Inc.	ANNX	ANX-005	Autoimmune Hemolytic Anemia (AIHA)	II	Phase II - Top-Line Results	06/30/2023	160969
Apexian Pharmaceuticals, Inc.		APX3330	Diabetic Macular Edema (Ophthalmology)	II	Phase II ZETA-1 - Top-Line Results	06/30/2023	165681
Applied Genetic Technologies Corporation	AGTC	AGTC-501	Retinitis Pigmentosa (RP) (Ophthalmology)	II/III	Phase II/III Vista - Top-Line Results	06/30/2023	167207
Aramis Biosciences, Inc.		A197	Dry Eye (Ophthalmology)	II	Phase II A197-CS-201 - Topline Results	06/30/2023	175380
Arcus Biosciences, Inc.	RCUS	Zimberelimab	Colorectal Cancer (CRC)	I/II	Phase Ib/II ARC-9 - Top-Line Results	06/30/2023	168469
argenx N.V.	ARGX	Efgartigimod (SC)	Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)	II	Phase II ADHERE - Top-Line Results	06/30/2023	174148
argenx N.V.	ARGX	Efgartigimod (SC)	Myasthenia Gravis (MG)	BLA	PDUFA for BLA - First Review	06/20/2023	180466
Arrowhead Pharmaceuticals, Inc.	ARWR	ARO-ANG3	Dyslipidemia / Hypercholesterolemia	IIb	Phase II Gateway - Top-Line Results	06/30/2023	182228
ARS Pharmaceuticals, Inc.		Neffy	Anaphylaxis	NDA	FDA Advisory Panel Meeting	05/11/2023	186086
ARS Pharmaceuticals, Inc.		Neffy	Anaphylaxis	NDA	FDA Advisory Panel Brief	05/09/2023	186087
Ascendis Pharma A/S	ASND	TransCon PTH	Hypoparathyroidism	NDA	PDUFA for NDA - First Review	04/30/2023	179887
Astellas Pharma, Inc.	4503:JP	Cresemba	Fungal Infections - Systemic	Approved	Phase III - Top-Line Results	05/31/2023	162737
Astellas Pharma, Inc.	4503:JP	Fezolinetant	Menopause (including Hormone Replacement Therapy [HRT])	NDA	PDUFA for NDA - First Review	05/22/2023	178339
AstraZeneca PLC	AZN	Lynparza	Prostate Cancer	Approved	FDA Advisory Panel Meeting	04/28/2023	185415
AstraZeneca PLC	AZN	Saphnelo	Systemic Lupus Erythematosus (SLE)	Approved	Phase III TULIP LTE - Top-Line Results	04/30/2023	157932
AstraZeneca PLC	AZN	Imfinzi	Hepatocellular (Liver) Cancer (HCC) (Including Secondary Metastases)	Approved	Phase III EMERALD-2 - Top-Line Results	06/30/2023	157904
AstraZeneca PLC	AZN	Imfinzi	Non-Small Cell Lung Cancer (NSCLC)	Approved	Phase III PACIFIC-5 - Top-Line Results	06/30/2023	154269
AstraZeneca PLC	AZN	Imfinzi	Small Cell Lung Cancer (SCLC)	Approved	Phase III ADRIATIC - Top-Line Results	06/30/2023	154274
AstraZeneca PLC	AZN	Imfinzi	Hepatocellular (Liver) Cancer (HCC) (Including Secondary Metastases)	Approved	Phase III EMERALD-1 - Top-Line Results	06/30/2023	154279
AstraZeneca PLC	AZN	Imfinzi	Ovarian Cancer	III	Phase III DuO-O - Top-Line Results	06/30/2023	147892
AstraZeneca PLC	AZN	Tagrisso	Non-Small Cell Lung Cancer (NSCLC)	Approved	Phase III FLAURA2 - Top-Line Results	06/30/2023	157890
AstraZeneca PLC	AZN	ALXN-1840	Wilson's Disease	III	Phase III - Pediatric Patients - Top-Line Results at AAN Meeting	04/25/2023	185442
AstraZeneca PLC	AZN	Cotadutide	Diabetic Nephropathy	II	Phase IIb CKD with T2DM - Top-Line Results	04/30/2023	168104

Biomedtracker Pharma Intelligence

Q2 2023 Large Impact Catalysts

Meddevicetracker Pharma Intelligence

Company	Symbol	Product	Indication	Phase	Catalyst Title	Expected End Date	Catalyst
AstraZeneca PLC	AZN	AZD5718	Chronic Kidney Disease (CKD)	II	Phase IIb Proteinuric CKD - Top-Line Results	06/30/2023	168102
AstraZeneca PLC	AZN	MEDI3506	Asthma	II	Phase II FRONTIER-3 - Top-Line Results	06/30/2023	168095
AstraZeneca PLC	AZN	MEDI3506	Atopic Dermatitis (Eczema)	II	Phase II Severe Atopic Dermatitis - Top-Line Results	06/30/2023	168096
AstraZeneca PLC	AZN	MEDI6570	Cardiovascular Disease	II	Phase IIb GOLDILOX - Top-Line Results	04/30/2023	168101
AstraZeneca PLC	AZN	Evusheld	COVID-19 Treatment	NDA	PDUFA for NDA - First Review (TACKLE)	05/31/2023	176993
AstraZeneca PLC	AZN	Farxiga/Zibotentan	Chronic Kidney Disease (CKD)	IIb	Phase IIb ZENITH-CKD - Top-Line Results	06/30/2023	180927
Atara Biotherapeutics, Inc.	ATRA	Tabelecleucel	Hematologic Cancer	III	Approval Decision (U.K.)	04/30/2023	181375
Athenex, Inc.	ATNX	Oral Paclitaxel	Breast Cancer	III	Phase II I-SPY 2 - Top-Line Results	06/30/2023	165901
Avadel Pharmaceuticals plc	AVDL	Lumryz	Narcolepsy	Approved	PDUFA for NDA - First Review	06/30/2023	165406
Avalo Therapeutics, Inc.	AVTX	AVTX-803	Metabolic - General	III	Pivotal Trial - Top-Line Results	06/30/2023	164853
Avinger, Inc.	AVGR	Pantheris	Peripheral Arterial Disease (PAD)	Approved	510(k) Approval Decision	04/30/2023	184217
Avita Medical, Inc.	RCEL	RECELL Device	Wound Healing	PMA	PMA Label Expansion Approval Decision	06/30/2023	183081
Avita Medical, Inc.	RCEL	RECELL Device	Vitiligo	PMA	PMA Approval Decision	06/30/2023	183221
AxioMed LLC		Freedom Lumbar Disc	Disc and Spine Repair	PMA	PMA Approval Decision	05/31/2023	173364
Axsome Therapeutics, Inc.	AXSM	AXS-12	Narcolepsy	III	Phase III SYMPHONY - Top-Line Results	06/30/2023	171569
Aziyo Biologics, Inc.	AZYO	CanGaroo Extracellular Matrix Envelope	Soft Tissue Repair	Approved	510(k) Clearance - Next Gen	06/30/2023	176745
AZTherapies, Inc.		ALZT-OP1	Alzheimer's Disease (AD)	III	Phase III COGNITE - Top-Line Results	06/30/2023	162512
Basilea Pharmaceutica Ltd.	BSLN	Derazantinib	Bladder Cancer	I/II	Phase II FIDES-02 - Updated Results	06/30/2023	162739
Bausch Health Companies Inc.	BHC	NovaTears	Dry Eye (Ophthalmology)	NDA	PDUFA for NDA - First Review	06/28/2023	178552
Bayer AG	BAYN	Mirena	Contraception	Approved	Regulatory - National Approval (EU)	04/30/2023	181203
BeiGene, Ltd.	BGNE	Tislelizumab	Esophageal Cancer	BLA	PDUFA for BLA - First Review	06/30/2023	171462
BeyondSpring Inc.	BYSI	Plinabulin	Non-Small Cell Lung Cancer (NSCLC)	III	Pre-NDA Meeting with FDA	04/30/2023	170874
BeyondSpring Inc.	BYSI	Plinabulin	Leukopenia / Neutropenia	III	Meeting with FDA	04/30/2023	173339
BioAge Labs, Inc.		BGE-175	COVID-19 Treatment	II	Phase II - Top-Line Results	05/31/2023	166917
BioAge Labs, Inc.		BGE-117	Anemia Due to Chronic Kidney Disease, Dialysis-Dependent	Development Outside U.S.	Phase II - Top-Line Results	05/31/2023	166455
Biocon, Ltd.	BIOS	Lextemy	Hepatocellular (Liver) Cancer (HCC) (Including Secondary Metastases)	BLA	PDUFA for 351(k) BLA - First Review	05/31/2023	165693
Biocon, Ltd.	BIOS	Lextemy	Colorectal Cancer (CRC)	BLA	PDUFA for 351(k) BLA - First Review	05/31/2023	158316
Biocon, Ltd.	BIOS	Lextemy	Non-Small Cell Lung Cancer (NSCLC)	BLA	PDUFA for 351(k) BLA - First Review	05/31/2023	158372
Biocon, Ltd.	BIOS	Lextemy	Renal Cell Cancer (RCC)	BLA	PDUFA for 351(k) BLA - First Review	05/31/2023	158373
Biocon, Ltd.	BIOS	Lextemy	Brain Cancer (Malignant Glioma; AA and glioblastoma (GBM))	BLA	PDUFA for 351(k) BLA - First Review	05/31/2023	158374
Biocon, Ltd.	BIOS	Lextemy	Cervical Cancer	BLA	PDUFA for 351(k) BLA - First Review	05/31/2023	158375
Biocon, Ltd.	BIOS	Lextemy	Ovarian Cancer	BLA	BsUFA for 351(k) BLA - First Review	05/31/2023	159259
Biocon, Ltd.	BIOS	Biosimilar Insulin Aspart (Viatris/Biocon)	Diabetes Mellitus, Type II	BLA	Meeting with FDA	04/30/2023	184636
BioCorRx Inc.	BICX	BICX-104	Opioid Use Disorder	I	Pre-NDA Meeting with FDA	06/30/2023	185878
Biogen, Inc.	BIIB	Tofersen	Amyotrophic Lateral Sclerosis (ALS)	NDA	PDUFA for NDA - First Review	04/25/2023	178940
Biogen, Inc.	BIIB	Aduhelm	Alzheimer's Disease (AD)	Approved	Japanese Approval Decision	04/30/2023	165212
BioLineRx Ltd.	BLRX	Aphexda	Pancreatic Cancer	IIb	Phase II w/Cemiplimab+Chemo - Top-Line Results	05/31/2023	163865
BioMarin Pharmaceutical Inc.	BMRN	Roctavian	Hemophilia A	BLA	Phase I/II 270-203 - Topline Results	06/30/2023	175240
BioMarin Pharmaceutical Inc.	BMRN	Roctavian	Hemophilia A	BLA	PDUFA for BLA - Second Review	06/30/2023	180694
BioMarin Pharmaceutical Inc.	BMRN	Roctavian	Hemophilia A	BLA	Phase III GENEr8-3 - Topline Results	04/30/2023	181714
Bioretec Ltd.		RemeOs	Bone Fractures and Mechanical Defects	De Novo	De Novo Approval Decision (US)	04/30/2023	177081

Biomedtracker Pharma Intelligence

Q2 2023 Large Impact Catalysts

Meddevicetracker Pharma Intelligence

Company	Symbol	Product	Indication	Phase	Catalyst Title	Expected End Date	Catalyst
BioSenic SA	BIOS	ALLOB	Bone Fractures and Mechanical Defects	I/II	Phase IIb Tibial Fracture - Top-Line Results	06/30/2023	164977
Biotest AG	BIO:GR	BT595	Primary Immunodeficiencies	III	Decentralized Approval Decision (Austria)	04/30/2023	179052
BioXcel Therapeutics, Inc.	BTAI	Igalmi	Schizophrenia	Approved	Phase III - SERENITY III - Top-Line Results	06/30/2023	182798
Blade Therapeutics, Inc		Cudetaxestat	Idiopathic Pulmonary Fibrosis (IPF)	I	Phase II - Top-Line Results	06/30/2023	166494
Braeburn Inc.		Brixadi	Opioid Use Disorder	Approved	PDUFA for NDA - Second Review	05/23/2023	183030
BrainsGate		Ischemic Stroke System (ISS)	Ischemic Stroke	PMA	PMA Approval Decision	06/30/2023	157520
Bristol Myers Squibb Company	BMY	Camzyos	Cardiomyopathy - Hypertrophic	Approved	Approval Decision (Europe)	05/31/2023	171908
Bristol Myers Squibb Company	BMY	Repotrectinib	Solid Tumors	I/II	Pre-NDA Meeting with the FDA	05/31/2023	175913
Byondis B.V.		Trastuzumab Duocarmazine	Breast Cancer	BLA	PDUFA for BLA – First Review	05/12/2023	178619
Calithera Biosciences, Inc.	CALA	Mivavotinib	Diffuse Large B-Cell Lymphoma (DLBCL) - NHL	II	Phase II - CX-659-401 (R/R non-GCB DLBCL) - Top-Line Results	06/30/2023	178322
Cardiawave S.A.		Valvosoft Platform	Cardiac Valve Surgery	Development Outside U.S.	CE Mark Approval	05/31/2023	145818
Cardiovascular Systems, Inc.	CSII	Innova Thrombectomy System	Peripheral Arterial Disease (PAD)	510(k)	510k Approval	04/30/2023	183582
Caris Life Sciences		MI Transcriptome Companion Diagnostic (CDx)	Solid Tumors	PMA	PMA Approval Decision	05/31/2023	159304
Caris Life Sciences		MI Exome Companion Diagnostic (CDx)	Solid Tumors	PMA	PMA Approval Decision	05/31/2023	159305
Celcuity, Inc.	CELC	CELx HER2 Signaling Function Test	Breast Cancer	Development	Phase II w/Capmatinib + Neratinib - Top-Line Results	05/31/2023	167869
CellTrans, Inc.		Lantidra	Diabetes Mellitus, Type I	BLA	PDUFA for BLA - First Review	06/30/2023	172673
Celltrion, Inc.	068270	Yuflyma	Juvenile Rheumatoid Arthritis	BLA	BsUFA for 351(k) BLA - First Review	06/30/2023	171219
Celltrion, Inc.	068270	Yuflyma	Axial Spondyloarthritis	Approved in Europe	BsUFA for 351(k) BLA - First Review	06/30/2023	171220
Celltrion, Inc.	068270	Yuflyma	Crohn's Disease	BLA	BsUFA for 351(k) BLA - First Review	06/30/2023	171221
Celltrion, Inc.	068270	Yuflyma	Psoriasis	BLA	BsUFA for 351(k) BLA - First Review	06/30/2023	171222
Celltrion, Inc.	068270	Yuflyma	Rheumatoid Arthritis (RA)	BLA	BsUFA for 351(k) BLA - First Review	06/30/2023	171223
Celltrion, Inc.	068270	Yuflyma	Ulcerative Colitis (UC)	BLA	BsUFA for 351(k) BLA - First Review	06/30/2023	171224
Celltrion, Inc.	068270	Yuflyma	Psoriatic Arthritis (PA)	BLA	BsUFA for 351(k) BLA - First Review	06/30/2023	171225
Cerevel Therapeutics Holdings, Inc.	CERE	Tavapadon	Parkinson's Disease (PD)	III	Phase III - TEMPO-4 - Top-line Results	06/30/2023	179783
Chiesi Farmaceutici S.p.A.		Elfabrio	Fabry's Disease	BLA	European Approval Decision	05/01/2023	175325
Chiesi Farmaceutici S.p.A.		Elfabrio	Fabry's Disease	BLA	PDUFA for BLA - Second Review	05/09/2023	182289
Cinclus Pharma Holding AB		Linaprazan Glurate	Gastroesophageal Reflux Disease (GERD)	II	Phase II - Dose Ranging (US/EU) - Top-Line Results	06/30/2023	171075
CLINUVEL PHARMACEUTICALS LIMITED	CLVLY	Scenesse	Skin Photodamage	Development Outside U.S.	Phase IIa CUV150 - Top-Line Results	05/31/2023	162545
CNS Pharmaceuticals, Inc.	CNSP	Berubicin	Brain Cancer (Malignant Glioma; AA and glioblastoma (GBM))	II	Phase II WHO Grade IV - Top-Line Results	06/30/2023	167623
Coherus BioSciences, Inc.	CHRS	Toripalimab	Non-Small Cell Lung Cancer (NSCLC)	III	Phase III - JS001-CT25-III-NSCLC (China) - Topline Results	06/30/2023	172332
Coherus BioSciences, Inc.	CHRS	Toripalimab	Small Cell Lung Cancer (SCLC)	Development Outside U.S.	Phase III - JUPITER-08 (China) - Topline Results	06/30/2023	172335
Collagen Solutions Plc	COS	ChondroMimetic	Cartilage and Joint Repair	Development Outside U.S.	CE Mark Approval	06/30/2023	140774
COMPASS Pathways	CMPS	COMP360	Major Depressive Disorder (MDD)	III	Phase II/III - Topline Results	06/30/2023	168398
Connect Biopharma Holdings Ltd.	CNTB	CBP-307	Ulcerative Colitis (UC)	II	Phase II - Top-Line Results	06/30/2023	183453
Covicept Therapeutics, Inc.		PSJ-539	COVID-19 Treatment	Development Outside U.S.	Phase II HALOS Top-Line Results	04/30/2023	172001
CSL Limited	CSL	VIT-2763	Sickle Cell Anemia	II	Phase IIa - Top-Line Results	06/30/2023	173547
Cyclo Therapeutics, Inc.	CYTH	Trappsol Cyclo	Niemann-Pick Disease	III	Phase III - TransportNPC Top Line Results	06/30/2023	170075

Biomedtracker Pharma Intelligence

Q2 2023 Large Impact Catalysts

Meddevicetracker Pharma Intelligence

Company	Symbol	Product	Indication	Phase	Catalyst Title	Expected End Date	Catalyst
Cytokinetics, Inc.	CYTK	Reldesemtiv	Amyotrophic Lateral Sclerosis (ALS)	III	Phase III COURAGE-ALS - Topline Results	05/31/2023	174421
Daiichi Sankyo Co., Ltd.	4568	Injectafer	Anemia in Heart Failure	III	Phase III HEART-FID - Top-Line Results	06/30/2023	162564
Daiichi Sankyo Co., Ltd.	4568	Quizartinib	Acute Myelogenous Leukemia (AML)	NDA	PDUFA for NDA - Second Review	04/24/2023	181490
Daiichi Sankyo Co., Ltd.	4568	Datopotamab Deruxtecan	Non-Small Cell Lung Cancer (NSCLC)	III	Phase III TROPION-LUNG01 - Top-Line Results	06/30/2023	176639
Daré Bioscience, Inc.	DARE	SST-6007	Female Sexual Arousal Disorder	II	Phase IIb RESPOND - Top-Line Results	06/30/2023	168010
Day One Biopharmaceuticals, LLC	DAWN	DAY101	Brain Cancer (Malignant Glioma; AA and glioblastoma (GBM))	III	Pre-NDA Meeting	06/30/2023	183888
Dermavant Sciences Inc.		Tapinarof	Atopic Dermatitis (Eczema)	III	Phase III ADORING 1 - Topline Data	05/31/2023	174242
Disc Medicine, Inc.	IRON	Bitopertin	Porphyria	II	Phase II BEACON - Top-Line Results	06/30/2023	179326
Eagle Pharmaceuticals, Inc.	EGRX	Barhemsys	Emesis	Approved	Approval Decision (Europe)	06/30/2023	171786
Eagle Pharmaceuticals, Inc.	EGRX	Barhemsys	Emesis	Approved	CHMP Opinion	05/31/2023	171789
Eagle Pharmaceuticals, Inc.	EGRX	Rapibloc	Dysrhythmia (Arrhythmia)	NDA	PDUFA for NDA - First Review	06/01/2023	177875
Edwards Lifesciences Corp.	EW	EVOQUE	Cardiac Valve Surgery	Development	PMA Approval Decision	06/30/2023	174194
Eiger BioPharmaceuticals, Inc.	EIGR	Zokinvy	Hepatitis D (HDV) (Antiviral)	III	Pre-NDA meeting with FDA	05/31/2023	183048
Elanix Biotechnologies AG	ELN	FirstCover	Burn Injury	Development Outside U.S.	CE Mark Approval	06/30/2023	140592
Elanix Biotechnologies AG	ELN	FirstCover	Wound Healing	Development Outside U.S.	CE Mark Approval	06/30/2023	140593
Eli Lilly and Company	LLY	Verzenio	Prostate Cancer	II/III	Phase II CYCLONE 1 - Top-Line Results at AACR	04/17/2023	185686
Eli Lilly and Company	LLY	Mirikizumab	Ulcerative Colitis (UC)	BLA	PDUFA for BLA - First Review	04/28/2023	176891
Eli Lilly and Company	LLY	Mirikizumab	Ulcerative Colitis (UC)	BLA	CHMP Opinion	06/30/2023	177679
Eli Lilly and Company	LLY	Donanemab	Alzheimer's Disease (AD)	III	Phase III TRAILBLAZER-ALZ 2 - Top-Line Results	06/30/2023	166877
Eloxx Pharmaceuticals, Inc.	ELOX	ELX-02	Alport Syndrome	II	Phase II Proof-of-Concept - Topline Results	06/30/2023	181805
Embera NeuroTherapeutics, Inc.		EMB-001	Cocaine Use Disorder	II	Phase II - Top-Line Results	06/30/2023	170280
Emerald Health Pharmaceuticals Inc.		EHP-101	Systemic Sclerosis	II	Phase IIa - Top-Line Results	04/30/2023	184290
Emergent BioSolutions, Inc.	EBS	AV7909	Anthrax Infection (Antibacterial)	BLA	PDUFA for BLA - First Review	04/30/2023	177189
Empirical Spine, Inc		LimiFlex Paraspinous Tension Band	Disc and Spine Repair	PMA	PMA Approval Decision (Module III)	06/30/2023	183281
Enanta Pharmaceuticals, Inc.	ENTA	EDP-235	COVID-19 Treatment	II	Phase II SPRINT - Top-Line Results	05/31/2023	182419
Endologix, Inc.		Nellix Endovascular Aneurysm Sealing System	Aortic Aneurysm	PMA	CE Mark Approval - Nellix CHEVAS	05/31/2023	118178
Endologix, Inc.		PQ Crossing Device	Peripheral Arterial Disease (PAD)	PMA	PMA Approval Decision	04/30/2023	181021
Enterome Bioscience		Sibofimloc	Crohn's Disease	II	Phase IIa - Top-Line Results	06/30/2023	162869
Enterome Bioscience		EO2463	Marginal Zone Lymphoma - NHL	I/II	Phase I/II SYDNEY - Top-Line Results at ASCO	06/30/2023	179771
EQRx, Inc.	EQRX	Ameile	Non-Small Cell Lung Cancer (NSCLC)	II	Approval Decision (U.K.)	04/30/2023	178149
EsoCap AG		ESO-101	Esophagitis	II	Phase II ACESO - Top-Line Data	06/30/2023	172404
Eton Pharmaceuticals, Inc.	ETON	DS-100	Drug Toxicity	NDA	PDUFA Approval	06/27/2023	185507
Eupraxia Pharmaceuticals Inc.	EPRX	EP-104IAR	Osteoarthritis	II	Phase II Efficacy and Safety - Top-Line Results	06/30/2023	170532
Evommune, Inc.		EVO101	Atopic Dermatitis (Eczema)	II	Phase IIa - Proof-of-Concept - Preliminary Data	06/30/2023	180664
ExCellThera Inc.		ECT001	Hematologic Cancer	II	Phase I/II Trial - Top-line Results	06/30/2023	182346
Exogenesis Corporation		Exogenesis Hernia Mesh	Hernia Repair	Approved	510(k) Approval Decision	04/30/2023	168272
Eyenovia, Inc.	EYEN	MydCombi	Other Ophthalmological Indications (Ophthalmology)	NDA	PDUFA for NDA - Second Review	05/08/2023	183087
Eyenovia, Inc.	EYEN	MicroLine	Refractive Errors (Ophthalmology)	III	Pre-NDA Meeting with FDA	06/30/2023	181432

Biomedtracker Pharma Intelligence

Q2 2023 Large Impact Catalysts

Meddevicetracker Pharma Intelligence

Company	Symbol	Product	Indication	Phase	Catalyst Title	Expected End Date	Catalyst
F2G Ltd		Olorofim	Fungal Infections - Systemic	NDA	PDUFA/Approval Decision (US)	06/17/2023	183238
Fennec Pharmaceuticals Inc.	FRX	Pedmark	Hearing Loss - Chemotherapy-Induced	Approved	Approval Decision (Europe)	06/30/2023	157709
FibroGen, Inc.	FGEN	Pamrevlumab	Duchenne Muscular Dystrophy (DMD)	III	Phase III LELANTOS-1 - Top-Line Results	06/30/2023	164820
First Wave BioPharma, Inc.	FWBI	FW-COV	COVID-19 Treatment	II	Phase II RESERVOIR (GI Infections) – Top-Line Results	05/31/2023	168268
Fresenius SE & Co. KGaA	FSNUY	Biosimilar Tocilizumab (Fresenius Kabi)	Rheumatoid Arthritis (RA)	BLA	PDUFA for Biosimilar 351(k) - First Review	06/30/2023	179464
Futura Medical plc		MED3000	Erectile Dysfunction (ED)	Approved in Europe	De Novo Approval Decision	06/30/2023	171693
Galderma S.A.		Dysport	Neurogenic Bladder	III	Approval Decision (Europe)	05/31/2023	178070
Galderma S.A.		Nemolizumab	Pruritus	III	Phase III OLYMPIA 1 - Results	06/30/2023	185656
Gamida Cell Ltd.	GMDA	Omidubicel	Bone Marrow Transplant and Stem Cell Transplant	BLA	PDUFA for BLA - First Review	05/01/2023	177917
Genmab A/S	GMAB	Epcoritamab	Diffuse Large B-Cell Lymphoma (DLBCL) - NHL	BLA	PDUFA for BLA - First Review	05/21/2023	181685
Gensco Pharma		Rizaport	Migraine and Other Headaches	NDA	PDUFA for NDA (4th Review)	04/17/2023	182114
Gilead Sciences, Inc.	GILD	Trodelvy	Non-Small Cell Lung Cancer (NSCLC)	III	Phase II TROPiCS-03 - Top-Line Results	04/30/2023	167361
Gilead Sciences, Inc.	GILD	Yescarta	Diffuse Large B-Cell Lymphoma (DLBCL) - NHL	Approved	Phase II ZUMA-24 - Top-Line Results	06/30/2023	183784
Glyscend Therapeutics, Inc.		GLY-200	Diabetes Mellitus, Type II	II	Phase II - Top-Line Results	06/30/2023	180450
GSK plc	GSK	Mosquirix (with MPL adjuvant)	Malaria	II	Phase II - Top-Line Results	06/30/2023	172564
GSK plc	GSK	EXXUA	Major Depressive Disorder (MDD)	NDA	PDUFA for NDA	06/23/2023	184280
GSK plc	GSK	Zejula	Prostate Cancer	NDA	Approval Decision (Europe)	05/01/2023	176872
GSK plc	GSK	Momelotinib	Myelofibrosis (MF)	NDA	PDUFA for NDA - First Review	06/16/2023	178218
GSK plc	GSK	Jesduvroq	Anemia Due to Chronic Kidney Disease, Dialysis-Dependent	Approved	CHMP Opinion	04/30/2023	175410
GSK plc	GSK	Jesduvroq	Anemia Due to Chronic Kidney Disease, Dialysis-Dependent	Approved	European Approval Decision	06/30/2023	175411
GSK plc	GSK	Jesduvroq	Anemia Due to Chronic Kidney Disease, Dialysis-Independent	NDA	CHMP Opinion	04/30/2023	175468
GSK plc	GSK	Jesduvroq	Anemia Due to Chronic Kidney Disease, Dialysis-Independent	NDA	European Approval Decision	06/30/2023	175469
GSK plc	GSK	Jesduvroq	Anemia Due to Chronic Kidney Disease, Dialysis-Independent	NDA	PDUFA for NDA - First Review	04/30/2023	176685
GSK plc	GSK	Bepirovirsen	Hepatitis B (HBV) Treatment (Antiviral)	III	Phase II B-Together - Top-Line Results	06/30/2023	179009
GSK plc	GSK	GSK3640254	HIV / AIDS	IIb	Phase IIb Treatment-Naive - Top-Line Results	06/30/2023	168939
GSK plc	GSK	Arexvy	Respiratory Syncytial Virus (RSV) Prevention	BLA	PDUFA for BLA - First Review	05/03/2023	181808
H. Lundbeck A/S	LUN	Aripiprazole 2-Month	Schizophrenia	NDA	PDUFA for NDA - First Review	04/27/2023	180264
H. Lundbeck A/S	LUN	Aripiprazole 2-Month	Bipolar Disorder	NDA	PDUFA for NDA - First Review	04/27/2023	180266
HanAll Biopharma Co., Ltd.	009420	HL036	Dry Eye (Ophthalmology)	III	Phase III VELOS-3 - Top-Line Results	06/30/2023	171951
Helixmith Co., Ltd.	084990	Engensis	Diabetic Peripheral Neuropathy (DPN)	III	Phase III REGAiN-1A - Top-Line Results	05/31/2023	170462
HEMA Biologics		SEVENFACT	Hemophilia A and B - General Clotting Products	Approved	Approval Decision (U.K.)	04/30/2023	178902
Hepion Pharmaceuticals, Inc.	HEPA	CRV431	Non-Alcoholic Steatohepatitis (NASH)	IIb	Phase II ALTITUDE NASH - Topline Results	06/30/2023	182900
Horizon Therapeutics plc	HZNP	Tepezza	Thyroid Eye Disease (TED)	Approved	Chronic TED - Top-Line Results	06/30/2023	164315
Horizon Therapeutics plc	HZNP	HZN-4920	Kidney Transplant Rejection	II	Phase II - Top-Line Results	04/30/2023	171938
Hugel Pharma Co, Ltd		Botulax	Wrinkles	BLA	PDUFA for BLA - Second Review	04/06/2023	170028
Human Immunology Biosciences, Inc.		Felzartamab	Membranous Nephropathy	II	Phase IIa newPLACE - Top-Line Results	05/31/2023	174540
Human Immunology Biosciences, Inc.		Felzartamab	Immunoglobulin A (IgA) Nephropathy (Berger's Disease)	II	Phase II IGNAZ - Top-Line Results	05/31/2023	174541
Humanigen, Inc.	HGEN	Lenzilumab	COVID-19 Treatment	III	Approval Decision (UK)	04/30/2023	171912
Humanigen, Inc.	HGEN	Lenzilumab	Graft vs. Host Disease (GVHD) - Treatment	Development Outside U.S.	Phase II/III RATinG Study - Interim Results	06/30/2023	178986
Hyloris Pharmaceuticals SA	HYL	Miconazole - DB	Candidiasis	Development Outside U.S.	Phase II - Top-Line Results	06/30/2023	172617

Biomedtracker Pharma Intelligence

Q2 2023 Large Impact Catalysts

Meddevicetracker Pharma Intelligence

Company	Symbol	Product	Indication	Phase	Catalyst Title	Expected End Date	Catalyst
IACTA Pharmaceuticals, Inc.		IC-265	Dry Eye (Ophthalmology)	II	Phase II - Top-Line Results (Parallel-Group Study)	05/31/2023	170704
Illumina, Inc.	ILMN	TruSight Oncology 500 Companion Diagnostic	Solid Tumors	PMA	PMA Approval Decision	06/30/2023	154357
ImmunityBio Inc.	IBRX	Anktiva	Bladder Cancer	BLA	PDUFA for BLA	05/23/2023	177561
ImmunoGen, Inc.	IMGN	Elahere	Ovarian Cancer	Approved	Phase III MIRASOL - Top-Line Results	04/30/2023	151718
Incyte Corporation	INCY	Jakafi	Myelofibrosis (MF)	Approved	Phase III POC - Top-Line Results (w/INCB00928)	06/30/2023	166008
Incyte Corporation	INCY	Jakafi	Myelofibrosis (MF)	Approved	Phase III POC - Top-Line Results (w/INCB57643)	06/30/2023	166010
Incyte Corporation	INCY	Jakafi	Graft vs. Host Disease (GVHD) - Treatment	Approved	Approval Decision (Japan)	05/31/2023	168856
Incyte Corporation	INCY	Opzelura	Vitiligo	Approved	Approval Decision (Europe)	05/01/2023	172525
Indivior plc	INDV	OPNT003	Opioid Use Disorder	NDA	PDUFA for NDA - First Review	05/22/2023	182531
Innovent Biologics, Inc.	1801	IBI310	Cervical Cancer	Development Outside U.S.	Phase II - Top-Line Results at AACR	04/17/2023	185897
Innoviva, Inc.	INVA	SUL-DUR	Acinetobacter-Specific Agents (Antibacterial)	NDA	PDUFA for NDA - First Review	05/29/2023	182778
Innoviva, Inc.	INVA	SUL-DUR	Acinetobacter-Specific Agents (Antibacterial)	NDA	FDA Advisory Panel Meeting	04/17/2023	185641
Innoviva, Inc.	INVA	SUL-DUR	Acinetobacter-Specific Agents (Antibacterial)	NDA	FDA Advisory Panel Brief	04/15/2023	185642
INOTREM S.A.		Nangibotide	COVID-19 Treatment	II	Phase IIa- CoviTrem1 - Top-Line Results	06/30/2023	166946
Inovio Pharmaceuticals, Inc.	INO	INO-4700	Antiviral - Miscellaneous Vaccines	II	Phase II MERS-201 - Top-Line Results	06/30/2023	171116
Inovio Pharmaceuticals, Inc.	INO	INO-4700	Antiviral - Miscellaneous Vaccines	II	Phase II MERS-201 - Top-Line Results	06/30/2023	171116
Insulet Corporation	PODD	Omnipod 5	Diabetes Mellitus, Type II	510(k)	510(k) Approval Decision	06/30/2023	185911
Intercept Pharmaceuticals, Inc.	ICPT	Ocaliva	Non-Alcoholic Steatohepatitis (NASH)	NDA	PDUFA for NDA - Second Review	06/22/2023	183369
Intercept Pharmaceuticals, Inc.	ICPT	Ocaliva	Non-Alcoholic Steatohepatitis (NASH)	NDA	FDA Advisory Panel Meeting	05/19/2023	185597
Intercept Pharmaceuticals, Inc.	ICPT	Ocaliva	Non-Alcoholic Steatohepatitis (NASH)	NDA	FDA Advisory Panel Brief	05/19/2023	185598
IntraBio Inc.		IB1001	Niemann-Pick Disease	III	Phase III - IB1001-301 - Top-Line Results	06/30/2023	182557
Intra-Cellular Therapies, Inc.	ITCI	Caplyta	Bipolar Disorder	Approved	Phase III Study 403 - Top-Line Results	05/31/2023	160369
Invivoscribe Technologies, Inc.		LeukoStrat CDx FLT3 Mutation Assay (Quizartinib Companion Diagnostic)	Acute Myelogenous Leukemia (AML)	PMA	PMA Supplemental Approval Decision	04/22/2023	181492
Ionis Pharmaceuticals, Inc.	IONS	IONIS-GHR-LRx	Acromegaly	II	Phase II OLE - Top-Line Results	04/30/2023	169376
Ionis Pharmaceuticals, Inc.	IONS	IONIS-GHR-LRx	Acromegaly	II	Phase II OL - Top-Line Results	05/31/2023	177397
Ipsen SA	IPSEY	Bylvay	Alagille Syndrome	NDA	PDUFA for sNDA - First Review	06/15/2023	183253
Iveric Bio	ISEE	Zimura	Stargardt Disease (Ophthalmology)	IIb	Phase IIb - STAR - Top-Line Results	05/31/2023	137980
Jazz Pharmaceuticals plc	JAZZ	Nabiximols	Neuromuscular Spasm and Spasticity	III	Phase III RELEASE MSS5 - Top-Line Results	05/31/2023	166124
Jazz Pharmaceuticals plc	JAZZ	Zepzelca	Small Cell Lung Cancer (SCLC)	Approved	Approval Decision (U.K.)	05/31/2023	179494
Johnson & Johnson	JNJ	JNJ-40411813	Seizure Disorders (Epilepsy)	II	Phase II - w/Levetiracetam - Top-Line Results	05/31/2023	169853
Johnson & Johnson	JNJ	Jcovden	COVID-19 Prevention	III	Supplemental Approval Decision (Europe)	04/30/2023	173643
Journey Medical Corp.	DERM	Minolira	Rosacea	III	Phase III - MVOR-01 - Top-Line Results	06/30/2023	179918
Journey Medical Corp.	DERM	Minolira	Rosacea	III	Phase III - MVOR-02 - Top-Line Results	06/30/2023	179920
Kane Biotech Inc.	KNE	coactiv+ Antimicrobial Hydrogel	Wound Healing	Development	510(k) Approval Decision	04/30/2023	181933
Kintor Pharmaceutical Ltd.	9939	Pruxelutamide	Prostate Cancer	II	Phase III - China (1L mCRPC) Top-Line Results	05/31/2023	156311
Kintor Pharmaceutical Ltd.	9939	KX-826	Androgenetic Alopecia	II	Phase II Male Alopecia (US) - Top-Line Results	06/30/2023	186091
Kissei Pharmaceutical Co., Ltd	4547	KPS-0373	Spinocerebellar Ataxia	Development Outside U.S.	Approval Decision (Japan)	05/31/2023	173804
Krystal Biotech, Inc.	KRYS	Vyjuvek	Epidermolysis Bullosa	BLA	PDUFA for BLA - First Review	05/19/2023	178280

Biomedtracker Pharma Intelligence

Q2 2023 Large Impact Catalysts

Meddevicetracker Pharma Intelligence

Company	Symbol	Product	Indication	Phase	Catalyst Title	Expected End Date	Catalyst
Kyowa Kirin Co., Ltd.	4151:JP	AVTX-002	Asthma	II	Phase II - PEAK - Top-Line Results	06/30/2023	174084
Laboratorios Hipra		COVID-19 Vaccine (HIPRA)	COVID-19 Prevention	Development Outside U.S.	Conditional Marketing Authorization (EU)	06/30/2023	176309
Laboratorios Salvat, S.A.		Clotrimazole	Ear Infections (Antibacterial)	III	PDUFA for NDA - First Review	06/30/2023	185981
Lava Therapeutics NV	LVTX	LAVA-051	Hematologic Cancer	I/II	Phase I/IIa - Updated Results (Expansion Cohorts)	06/30/2023	172216
Les Laboratoires Servier		Tibsovo	Biliary Tract Cancer	Approved	Approval Decision (Europe)	05/01/2023	175722
Les Laboratoires Servier		Tibsovo	Acute Myelogenous Leukemia (AML)	Approved	Approval Decision (Europe)	05/01/2023	175723
Lexicon Pharmaceuticals, Inc.	LXRX	Zynquista	Chronic Heart Failure - Preserved Ejection Fraction (Chronic HFpEF)	NDA	PDUFA for First Review	05/31/2023	177844
Lexicon Pharmaceuticals, Inc.	LXRX	Zynquista	Chronic Heart Failure - Reduced Ejection Fraction (Chronic HFrEF)	NDA	PDUFA for First Review	05/31/2023	177925
Lipella Pharmaceuticals Inc.	LIPO	LP-10	Interstitial Cystitis / Painful Bladder Syndrome	II	Phase II - Top-Line Results at AUA	04/30/2023	185810
Lipocine Inc.	LPCN	LPCN 1154	Neurology - Other	II	Phase II - Top-Line Results	06/30/2023	169983
Lipocine Inc.	LPCN	LPCN 1154	Neurology - Other	II	PK Bridge Study - Top-Line Results	06/30/2023	180342
Lisata Therapeutics, Inc.	LSTA	Honedra	Peripheral Arterial Disease (PAD)	Development Outside U.S.	Japanese Approval Decision	06/30/2023	167865
LivaNova PLC	LIVN	aura6000	Sleep Apnea	IDE	PMA Approval Decision (US)	06/30/2023	142711
Lumicell Inc.		Lumicell Direct Visualization System	Breast Cancer - Imaging	IDE	PMA Filing	06/30/2023	185928
Lupin Limited	LPC	Alinia	COVID-19 Prevention	III	Phase III Healthcare Workers - Top-Line Results	05/31/2023	168552
Lupin Limited	LPC	Biosimilar Pegfilgrastim (Lupin)	Leukopenia / Neutropenia	BLA	PDUFA for 351(k) Biosimilar	05/31/2023	173783
Lutris Pharma		LUT014	Acne	II	Phase II L-02-01 - Top-Line Results	04/30/2023	168907
Luye Pharma Group, Ltd.	2186	LY03005	Major Depressive Disorder (MDD)	NDA	PDUFA for NDA - First Review	06/30/2023	156247
MaaT Pharma SA		MaaT013	Graft vs. Host Disease (GVHD) - Treatment	Development Outside U.S.	Phase III ARES - Topline Results	06/30/2023	176105
Mallinckrodt plc	MNKKQ	CPP-1X/Sulindac	Familial Adenomatous Polyposis (FAP)	NDA	PDUFA for NDA - First Review	06/30/2023	160833
Marinus Pharmaceuticals, Inc.	MRNS	Ztalmy	Seizure Disorders (Epilepsy)	Approved	CHMP Opinion	05/31/2023	171990
Marinus Pharmaceuticals, Inc.	MRNS	Ztalmy	Seizure Disorders (Epilepsy)	Approved	European Approval Decision	05/31/2023	171991
MC2 Therapeutics A/S		Wynzora Cream	Psoriasis	Approved	Approval Decision (Europe)	04/30/2023	161146
MC2 Therapeutics A/S		Wynzora Cream	Psoriasis	Approved	CHMP Opinion	06/30/2023	161152
medac GmbH		Ovastat	Bone Marrow Transplant and Stem Cell Transplant	NDA	PDUFA for NDA - Second Review	06/30/2023	176738
MediWound Ltd.	MDWD	MW005	Skin Cancer - Basal Cell Carcinoma (BCC)	II	Phase II - Top-Line Results	06/30/2023	167346
Medtronic plc	MDT	Symplicity Renal Denervation System	Hypertension (Systemic)	PMA	US Approval Decision	06/30/2023	163437
Medtronic plc	MDT	MiniMed 780G	Diabetes Mellitus, Type I	PMA	PMA Approval	06/30/2023	168061
Melinta Therapeutics, Inc.	MLNT	Solithera	Respiratory Tract Infections (Excluding Pneumonia) (Antibacterial)	Development Outside U.S.	Approval Decision (Japan)	05/31/2023	176394
Melinta Therapeutics, Inc.	MLNT	Solithera	Ear Infections (Antibacterial)	Development Outside U.S.	Approval Decision (Japan)	05/31/2023	176396
Melinta Therapeutics, Inc.	MLNT	REZZAYO	Fungal Infections - Systemic	III	Phase III ReSPECT - Top-Line Results	04/30/2023	141375
Merck & Co., Inc.	MRK	MK-7264	Chronic Cough	III	CHMP Opinion	05/31/2023	171021
Merck & Co., Inc.	MRK	MK-7264	Chronic Cough	III	Approval Decision (Europe)	04/30/2023	171022
Merck & Co., Inc.	MRK	Keytruda	Diffuse Large B-Cell Lymphoma (DLBCL) - NHL	Approved	Phase IIb VITALIZE - Top-Line Results	04/30/2023	174550
Merck & Co., Inc.	MRK	Lagevrio	COVID-19 Treatment	II/III	Approval Decision (Japan)	06/30/2023	173421
Merck & Co., Inc.	MRK	Lagevrio	COVID-19 Treatment	II/III	European Approval Decision (Rolling Review)	04/30/2023	173220
Merck KGaA	MKKGY	Pamiparib	Ovarian Cancer	Approved in other than U.S./E.U.	Phase III Recurrent Platinum-Sensitive (China) - Top-Line Results	04/30/2023	155778
Merck KGaA	MKKGY	Bintrafusp Alfa	Cervical Cancer	II	Phase II - INTR@PID CERVICAL 017 - Topline Data	04/30/2023	171236

Biomedtracker Pharma Intelligence

Q2 2023 Large Impact Catalysts

Meddevicetracker Pharma Intelligence

Company	Symbol	Product	Indication	Phase	Catalyst Title	Expected End Date	Catalyst
Minoryx Therapeutics		Leriglitazone	Adrenoleukodystrophy	II/III	Phase II NEXUS - Top-Line Results	06/30/2023	159696
Mirati Therapeutics, Inc.	MRTX	Sitravatinib	Non-Small Cell Lung Cancer (NSCLC)	III	Phase III - Interim ORR Analysis	06/30/2023	148334
Mirati Therapeutics, Inc.	MRTX	Krazati	Colorectal Cancer (CRC)	III	Phase III w/Cetuximab - Top-Line Results	06/30/2023	166032
Moderna, Inc.	MRNA	Spikevax	COVID-19 Prevention	Approved	Phase III 6 Months - 5 Years - Top-Line Results	04/30/2023	183206
MyMD Pharmaceuticals, Inc.		MYMD-1	Major Depressive Disorder (MDD)	IND	Phase II - Top-Line Results	05/31/2023	170901
NanoCarrier Co., Ltd.	4571	ENT103	Ear Infections (Antibacterial)	Development Outside U.S.	Phase III Efficacy (Japan) - Top-Line Results	04/30/2023	175142
Nanoscope Therapeutics		Sonpiretigene Isteparvovec	Retinitis Pigmentosa (RP) (Ophthalmology)	IIb	Phase II RESTORE (US) - Top-Line Results	06/30/2023	171795
Neovasc Inc.	NVCN	Tiara TA	Cardiac Valve Surgery	Development	TIARA TA - CE Mark Approval	06/30/2023	169946
NF Gamaleya NITsEM		Sputnik Light	COVID-19 Prevention	Approved in other than U.S./E.U.	Phase III - SPUTNIK-LIGHT - Top-Line Results	04/30/2023	170452
NGM Biopharmaceuticals, Inc.	NGM	Aldafermin	Non-Alcoholic Steatohepatitis (NASH)	IIb	Phase II ALPINE 4 - Top-Line Results	06/30/2023	175873
Nobelpharma Co., Ltd.		Hyftor	Tuberous Sclerosis Complex (TSC)	Approved	EU Approval Decision	05/01/2023	185301
Novaliq GmbH		CyclASol	Dry Eye (Ophthalmology)	NDA	PDUFA for NDA - First Review	06/08/2023	179288
Novartis AG	NVS	Entresto	Chronic Heart Failure - Reduced Ejection Fraction (Chronic HFrEF)	Approved	Phase II/III PANORAMA HF - Top-Line Results	04/30/2023	150227
Novartis AG	NVS	Cosentyx	Axial Spondyloarthritis	Approved	Phase III INVIGORATE 1 - Topline Results	04/30/2023	166847
Novartis AG	NVS	Cosentyx	Psoriatic Arthritis (PA)	Approved	Phase III INVIGORATE 2 - Topline Results	04/30/2023	166848
Novartis AG	NVS	Cosentyx	Dermatology	II	Phase II PRELUDE - Topline Results	04/30/2023	166850
Novartis AG	NVS	Cosentyx	Axial Spondyloarthritis	Approved	Phase III SURPASS - Topline Results	04/30/2023	151094
Novartis AG	NVS	Cosentyx	Hidradenitis Suppurativa	NDA	Supplemental CHMP Opinion Results	04/30/2023	178810
Novartis AG	NVS	Cosentyx	Hidradenitis Suppurativa	NDA	Supplemental EU Approval Decision	06/30/2023	178811
Novartis AG	NVS	Tabrecta	Non-Small Cell Lung Cancer (NSCLC)	Approved	Phase III GeoMETRY-III - Topline Results	05/31/2023	167612
Novartis AG	NVS	Piqray	Proteus Syndrome	Approved	Phase II EPIK-P2 – Top-Line Results	06/30/2023	170833
Novartis AG	NVS	Beovu	Diabetic Macular Edema (Ophthalmology)	Approved	Approval Decision (Japan)	04/30/2023	172162
Novartis AG	NVS	LIK066	Non-Alcoholic Steatohepatitis (NASH)	II	Phase II ELIVATE - Topline Results	06/30/2023	166851
Novartis AG	NVS	Hyrimoz	Ulcerative Colitis (UC)	Approved	Supplemental Approval Decision (Europe)	04/07/2023	178227
Novartis AG	NVS	Hyrimoz	Crohn's Disease	Approved	Supplemental Approval Decision (Europe)	04/07/2023	178234
Novartis AG	NVS	Hyrimoz	Rheumatoid Arthritis (RA)	Approved	Supplemental Approval Decision (Europe)	04/07/2023	178235
Novartis AG	NVS	Hyrimoz	Uveitis (Ophthalmology)	Approved in Europe	Supplemental Approval Decision (Europe)	04/07/2023	178236
Novartis AG	NVS	Hyrimoz	Psoriasis	Approved	Supplemental Approval Decision (Europe)	04/07/2023	178214
Novartis AG	NVS	LJN452	Non-Alcoholic Steatohepatitis (NASH)	IIb	Phase II ELIVATE - Topline Results	06/30/2023	166852
Novartis AG	NVS	Zolgensma	Spinal Muscular Atrophy	Approved	Phase III STR1VE (Asia Pacific) - Updated Results	04/30/2023	166875
Novartis AG	NVS	Iscalimab	Systemic Lupus Erythematosus (SLE)	II	Phase II VAY736 and CFZ533 - Top-Line Results	06/30/2023	155763
Novartis AG	NVS	Iscalimab	Hidradenitis Suppurativa	II	Phase II LYS006/CFZ533 - Top-Line Results	04/30/2023	155764
Novartis AG	NVS	BAT-1706	Non-Small Cell Lung Cancer (NSCLC)	BLA	CHMP Opinion	04/30/2023	164703
Novartis AG	NVS	BAT-1706	Non-Small Cell Lung Cancer (NSCLC)	BLA	European Approval Decision	04/30/2023	164705
Novartis AG	NVS	BAT-1706	Non-Small Cell Lung Cancer (NSCLC)	BLA	PDUFA for 351(k) BLA - First Review	04/30/2023	165578
Novartis AG	NVS	Biosimilar Trastuzumab (EirGenix/Novartis)	Breast Cancer	III	Approval Decision (Europe)	05/31/2023	173805
Novartis AG	NVS	Biosimilar Trastuzumab (EirGenix/Novartis)	Breast Cancer	III	CHMP Opinion	04/30/2023	173803
Novartis AG	NVS	Biosimilar Natalizumab (Sandoz)	Multiple Sclerosis (MS)	BLA	PDUFA for BLA - First Review	06/30/2023	178866

Biomedtracker Pharma Intelligence

Q2 2023 Large Impact Catalysts

Meddevicetracker Pharma Intelligence

Company	Symbol	Product	Indication	Phase	Catalyst Title	Expected End Date	Catalyst
Novavax, Inc.	NVAX	Nuvaxovid	COVID-19 Prevention	Approved in Europe	Phase II/III Hummingbird - Top-Line Results	06/30/2023	179194
Novo Nordisk A/S	NVO	NovoSeven	Acute Hemorrhage	Development Outside U.S.	Approval Decision (Europe) - Pospartum Hemorrhage	06/30/2023	176743
Novo Nordisk A/S	NVO	Ozempic	Diabetes Mellitus, Type II	Approved	Phase II Overweight - Top-Line Results	06/30/2023	182602
Novo Nordisk A/S	NVO	Rybelsus	Obesity	III	Phase IIIa OASIS 1 - Top-Line Results	06/30/2023	182636
Novo Nordisk A/S	NVO	PYY1875	Obesity	II	Phase II w/ Semaglutide - Top-Line Results	06/30/2023	182563
Novo Nordisk A/S	NVO	Etavopivat	Sickle Cell Anemia	II/III	HIBISCUS - Top-Line Results	06/30/2023	176318
Novo Nordisk A/S	NVO	Etavopivat	Sickle Cell Anemia	II/III	Phase II - w/Thalassemia or Sickle Cell Disease - Top-Line Results	06/30/2023	176322
Novo Nordisk A/S	NVO	Wegovy	Obesity	Approved	Supplemental Approval (Japan)	06/30/2023	175511
Omeros Corporation	OMER	Narsoplimab	Transplant-Associated Thrombotic Microangiopathy (TA-TMA)	BLA	PDUFA for BLA - Second Review	06/30/2023	174813
Onxeo SA	ONXEO: FP	AsiDNA	Ovarian Cancer	I/II	Phase Ib/II - Top-Line Results	05/31/2023	157208
Ophirex, Inc		Varespladib methyl	Neurology - Other	II	Phase II BRAVO - Top-Line Results	04/30/2023	177202
Orbus Therapeutics, Inc.		Eflornithine (Orbus)	Brain Cancer (Malignant Glioma; AA and glioblastoma (GBM))	III	Phase III - Top-Line Results	06/30/2023	167515
Orchard Therapeutics Limited	ORTX	OTL-200	Metachromatic Leukodystrophy	III	Approval Decision - Swissmedic	06/30/2023	182801
Orchard Therapeutics Limited	ORTX	OTL-200	Metachromatic Leukodystrophy	III	Meeting with FDA	04/30/2023	176191
Oryzon Genomics S.A.	ORY	Vafidemstat	Phelan-McDermid Syndrome (PMS)	Development Outside U.S.	Pilot Study - Topline Results	05/31/2023	170154
Otsuka Holdings Co., Ltd.	4578	Rexulti	Neuropsychiatric Symptoms in Alzheimer's Disease	NDA	Psychopharmacologic Drugs Advisory Committee meeting	04/30/2023	183708
Otsuka Holdings Co., Ltd.	4578	Rexulti	Neuropsychiatric Symptoms in Alzheimer's Disease	NDA	PDUFA for sNDA/sBLA	05/10/2023	183709
Otsuka Holdings Co., Ltd.	4578	Paradise System	Hypertension (Systemic)	PMA	PMA Approval Decision	05/31/2023	182735
Palatin Technologies, Inc.	PTN	PL-8177	Ulcerative Colitis (UC)	II	Phase II PL8177-205 - Top-Line Results	06/30/2023	157207
Palatin Technologies, Inc.	PTN	PL-9643	Dry Eye (Ophthalmology)	III	Phase III MELODY-1 - Top-Line Results	06/30/2023	170252
PaxMedica, Inc.	PXMD	PAX-101	Anti-Parasitic and Anti-Protozoal	III	Phase III - HAT-301 - Top-Line Data	06/30/2023	182171
Perrigo Company PLC	PRGO	Opill	Contraception	Approved	FDA Advisory Panel Meeting	05/10/2023	180224
Perrigo Company PLC	PRGO	Opill	Contraception	Approved	FDA Advisory Panel Brief	05/08/2023	180225
Pfizer Inc.	PFE	Etrasimod	Alopecia Areata	II	Phase II - Top-Line Results	06/30/2023	157101
Pfizer Inc.	PFE	Ritlecitinib	Alopecia Areata	NDA	PDUFA for NDA - First Review	06/30/2023	179089
Pfizer Inc.	PFE	Prevnar 20	Pneumococcal (Streptococcus pneumoniae) Vaccines (Antibacterial)	Approved	Phase III HEALTHY INFANTS - Top Line Results	06/30/2023	174721
Pfizer Inc.	PFE	Abrysvo	Respiratory Syncytial Virus (RSV) Prevention	BLA	PDUFA for BLA (First Review)	05/31/2023	182980
Pfizer Inc.	PFE	GBT601	Sickle Cell Anemia	II/III	Phase II/III GBT601 - Top-Line Results	06/30/2023	178419
Pfizer Inc.	PFE	Paxlovid	COVID-19 Treatment	NDA	Phase III EPIC-Peds - Top-Line Results	06/30/2023	177231
Pfizer Inc.	PFE	Paxlovid	COVID-19 Treatment	NDA	Approval Decision (Europe)	04/04/2023	184535
Pfizer Inc.	PFE	Paxlovid	COVID-19 Treatment	NDA	PDUFA for NDA - First Review	05/31/2023	178445
Phathom Pharmaceuticals, Inc.	PHAT	Takecab	Esophagitis	NDA	Meeting with FDA	05/31/2023	184894
Phathom Pharmaceuticals, Inc.	PHAT	Takecab	H. pylori Infection	Approved	Meeting with FDA	05/31/2023	184895
Pillar Biosciences, Inc.		oncoReveal Dx	Solid Tumors	PMA	PMA Supplemental Approval	06/30/2023	178740
PolyPid Ltd.	PYPD	D-PLEX	Intra-Abdominal Infections (Antibacterial)	III	Phase III SHIELD I - Updated Results	06/30/2023	179977
Poxel SA	POXEL	PXL065	Adrenoleukodystrophy	Preclinical	Phase IIa - PoC Study - Top-Line Results	04/30/2023	170465
Prestige Biopharma Pte. Ltd.		Tuznue	Breast Cancer	Development Outside U.S.	Meeting with FDA (BLA Pre-submission)	04/30/2023	181173
Prilenia Therapeutics Development Ltd.		TV-7820	Huntington's Disease	III	Phase III PROOF-HD - Top-Line Results	06/30/2023	172325

Biomedtracker Pharma Intelligence

Q2 2023 Large Impact Catalysts

Meddevicetracker Pharma Intelligence

Company	Symbol	Product	Indication	Phase	Catalyst Title	Expected End Date	Catalyst
PTC Therapeutics, Inc.	PTCT	PTC-743	Friedreich's Ataxia	II/III	Phase II/III - Top-Line Results	06/30/2023	167238
PTC Therapeutics, Inc.	PTCT	PTC-743	Mitochondrial Respiratory-Chain Diseases	II/III	Phase II/III MIT-E - Top-Line Results	06/30/2023	167239
PTC Therapeutics, Inc.	PTCT	PTC518	Huntington's Disease	II	Phase II PIVOT-HD - Top-Line Results	06/30/2023	177588
Puma Biotechnology, Inc.	PBYI	Nerlynx	Cervical Cancer	II	Pre-NDA Meeting with FDA	05/31/2023	155818
Puma Biotechnology, Inc.	PBYI	Nerlynx	Breast Cancer	Approved	Pre-NDA Meeting with FDA	06/30/2023	155831
Puma Biotechnology, Inc.	PBYI	Nerlynx	Breast Cancer	Approved	Phase II FACT 2 - Top-Line Results	06/30/2023	146298
PureTech Health plc	PRTC	LYT-100	Edema	II	Phase IIa - Top-Line Results	06/30/2023	165232
Radius Health, Inc.		Tymlos	Osteoporosis / Osteopenia	Approved	Approval Decision (U.K.)	05/31/2023	178078
Rain Oncology Inc.	RAIN	Milademetan	Sarcoma	III	Phase III MANTRA - Top-Line Results	05/31/2023	168237
Rain Oncology Inc.	RAIN	Milademetan	Sarcoma	III	Phase II - Top-Line Results	05/31/2023	168239
Reata Pharmaceuticals, Inc.	RETA	Imbarkyd	Diabetic Nephropathy	Development Outside U.S.	Phase III AYAME - Top-Line Results	06/30/2023	144253
Reata Pharmaceuticals, Inc.	RETA	Imbarkyd	Alport Syndrome	III	Approval Decision (Japan)	05/31/2023	170705
Rediscovery Life Sciences		Suramin	Acute Kidney Injury (AKI)/Acute Renal Failure (ARF)	II	Phase II - Top-Line Results	06/30/2023	162884
Regeneron Pharmaceuticals, Inc.	REGN	REGEN-COV	COVID-19 Prevention	BLA	PDUFA for BLA - First Review (Non-Hospitalized)	06/30/2023	171136
ReShape Lifesciences, Inc.	RSLS	ReShape Vest	Obesity	Development	CE Mark Approval	06/30/2023	118696
Reven Pharmaceuticals, Inc.		Rejuveinix	COVID-19 Treatment	I/II	Phase II COVID-19 ARDS - Topline Results	06/30/2023	163644
Revolo Biotherapeutics		IRL201104	Allergy	Development Outside U.S.	Phase IIa - Top-Line Results	06/30/2023	183614
Roche Holding AG	RHHBY	Actemra (Subcutaneous)	Rheumatoid Arthritis (RA)	Approved	PDUFA for Biosimilar 351(k) - First Review	06/30/2023	179465
Roche Holding AG	RHHBY	OpRegen	Dry Age-Related Macular Degeneration (Dry AMD)/Geographic Atrophy (Ophthalmology)	I/II	Phase IIa GR44251 - Top-Line Results at RCGT	04/21/2023	185899
SanBio Co., Ltd	4592	SB623	Traumatic Brain Injury (TBI)	II	Approval Decision (Japan)	06/30/2023	175489
Saniona AB	SANION	Tesomet	Prader-Willi Syndrome	IIb	Phase IIb - Top-Line Results	06/30/2023	173883
Sanofi	SNY	SAR408701	Non-Small Cell Lung Cancer (NSCLC)	III	Phase II CARMEN-LC04 - Top-Line Results	04/30/2023	160335
Sanofi	SNY	SAR408701	Non-Small Cell Lung Cancer (NSCLC)	III	Phase II CARMEN-LC05 - Top-Line Results	04/30/2023	160337
Sarepta Therapeutics, Inc.	SRPT	SRP-9001	Duchenne Muscular Dystrophy (DMD)	BLA	PDUFA for BLA - First Review	05/29/2023	180672
Sarepta Therapeutics, Inc.	SRPT	SRP-9001	Duchenne Muscular Dystrophy (DMD)	BLA	FDA Advisory Panel Meeting	05/29/2023	185804
Sarepta Therapeutics, Inc.	SRPT	SRP-9001	Duchenne Muscular Dystrophy (DMD)	BLA	FDA Advisory Panel Brief	05/29/2023	185805
Secura Bio, Inc.		Copiktra	Peripheral T-Cell Lymphoma (PTCL) - NHL	II	Phase II PRIMO - Top-Line Results	05/31/2023	175133
Sedana Medical AB	SEDANA	Sedaconda	Drug Delivery Technology	Development	Approval Decision (UK)	04/30/2023	175255
SELLAS Life Sciences	SLS	Zeltherva	Acute Myelogenous Leukemia (AML)	III	Phase III - Top-Line	05/31/2023	129140
Seres Therapeutics,	MCRB	SER-109	Clostridium difficile-Associated	BLA	PDUFA for BLA - 1st	04/26/2023	180091
Shionogi & Co. Ltd.	4507	Fetroja	Urinary Tract and Reproductive Tract	Approved	Approval Decision (Japan)	06/30/2023	175997
Shionogi & Co. Ltd.	4507	S-217622	COVID-19 Treatment	III	Approval Decision (Japan)	05/31/2023	175360
Simcere Pharmaceutical Group	2096	Sanbexin	Ischemic Stroke	Approved in other than U.S./E.U.	Phase III - Top-Line Results	06/30/2023	182987
SK Bioscience		GBP510	COVID-19 Prevention	Approved in	European Approval	06/30/2023	179558
Soligenix, Inc.	SNGX	HyBryte	Cutaneous T-Cell Lymphoma (CTCL) -	III	Type A Meeting with FDA	04/30/2023	185000
Sorrento Therapeutics,	SRNEQ	Abivertinib	Non-Small Cell Lung Cancer (NSCLC)	III	Pre-NDA Meeting	05/31/2023	179645
Sorrento Therapeutics,	SRNEQ	COVI-DROPS	COVID-19 Treatment	II	Phase II US - Topline	06/30/2023	174784
Spruce Biosciences,	SPRB	Tildacerfont	Polycystic Ovary Syndrome (PCOS)	II	Phase II - Top-Line	06/30/2023	171202
Stealth		Elamipretide (Systemic	Cardiomyopathy - Dilated	III	Meeting with FDA	06/30/2023	178153
Sterna Biologicals		SB010	Asthma	II	Phase IIa POC Study-	04/30/2023	173221
Sumitovant Biopharma		Gemtesa	Overactive Bladder (OAB)	Approved	Phase III COURAGE - Top-	05/31/2023	151491
Sun Pharmaceutical	SUNP	MM-II	Osteoarthritis Pain	IIb	Phase IIb - CLR_17_17 -	06/30/2023	165516
Surface Oncology, Inc.	SURF	SRF388	Non-Small Cell Lung Cancer (NSCLC)	II	Phase II w/Pembrolizumab	06/30/2023	181822
Surface Oncology, Inc.	SURF	SRF388	Hepatocellular (Liver) Cancer (HCC)	II	Phase II - Top-Line	04/30/2023	171953
Swedish Orphan	SOBI	Doptelet	Liver Failure / Cirrhosis	Development	Approval Decision (Chronic	06/30/2023	184134
Swedish Orphan	SOBI	Gamifant	Histiocytosis	Approved	Phase III EMERALD -	06/30/2023	171949
Takeda	TAK	Livtencity	Cytomegalovirus (CMV) Infection	Approved	Phase III 302 (Wild Type) -	05/31/2023	155440
Takeda	TAK	TAK-003	Dengue Fever - Vaccines and	BLA	PDUFA for BLA - First	06/30/2023	182530
Takeda	TAK	TAK-951	Emesis	II	Phase II PONV - Top-Line	04/30/2023	170819

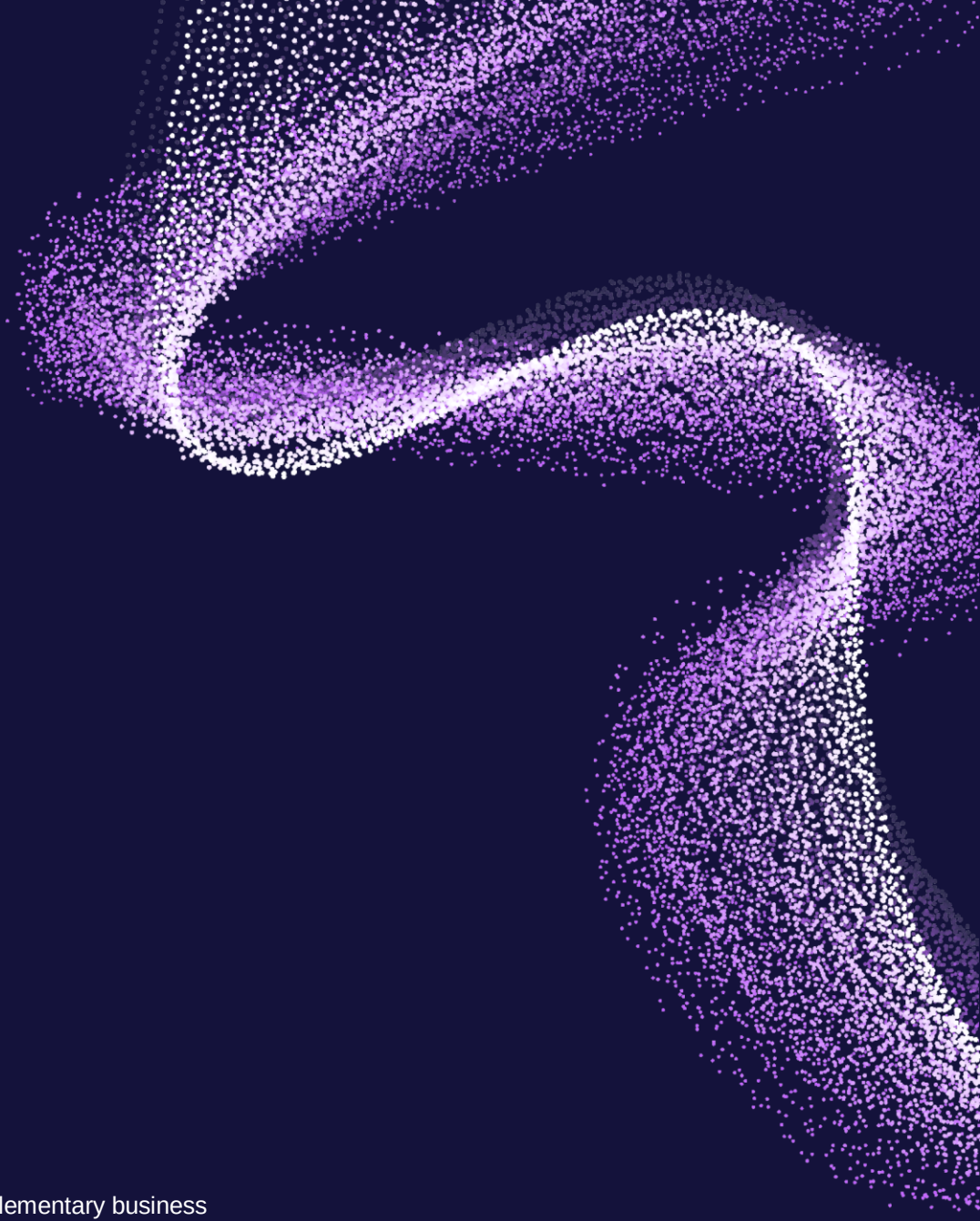
Biomedtracker Pharma Intelligence

Q2 2023 Large Impact Catalysts

Meddevicetracker Pharma Intelligence

Company	Symbol	Product	Indication	Phase	Catalyst Title	Expected End Date	Catalyst
TearSolutions, Inc.		Lacripep	Dry Eye (Ophthalmology)	I/II	Phase III General DED -	06/30/2023	175179
Teijin Medical		MT-Mag	Chronic Heart Failure - Reduced Ejection	Development	CE Mark Approval	05/31/2023	140912
Tenax Therapeutics, Inc.	TENX	Imatinib	Pulmonary Arterial Hypertension (PAH) and Pulmonary Hypertension (PH)	II/III	Phase II/III PK Study - Top-Line Results	06/30/2023	172019
Teva Pharmaceutical	TEVA	Uzedy	Schizophrenia	NDA	PDUFA for NDA - Second	06/30/2023	181882
TFF Pharmaceuticals,	TFFP	Tacrolimus Inhalation	Lung Transplant Rejection	Development	Phase II TFF-T2-001 - Top-	06/30/2023	181783
Tonix Pharmaceuticals	TNXP	Tonmya	Fibromyalgia	III	Phase III RESILIENT -	06/30/2023	181217
Trevena Inc.	TRVN	TRV027	COVID-19 Treatment	I	Phase II/III - ACTIV-4d	05/31/2023	171814
Tyber Medical, LLC.		Tyber Foot and Ankle	Bone Fractures and Mechanical Defects	Approved	CE Mark Approval	06/30/2023	163647
UCB S.A.	UCB	Bimzelx	Psoriasis	BLA	PDUFA for BLA - Second	06/30/2023	182538
UCB S.A.	UCB	Rozanolixizumab	Myasthenia Gravis (MG)	BLA	PDUFA - First Review	06/30/2023	183686
Ultimovacs ASA	ULTIMO	UV1	Melanoma	II	Phase II INITIUM - Top-	06/30/2023	163070
Ultragenyx	RARE	UX053	Glycogen Storage Disease (GSD)	II	Phase I/II - GSD III -	06/30/2023	174287
Vaxcyte, Inc	PCVX	VAX-24	Pneumococcal (Streptococcus	II	Phase II - w/Prevnar 20	06/30/2023	178515
VectivBio Holding AG	VECT	Apraglutide	Short Bowel Syndrome (SBS)	III	Phase II STARS Nutrition -	06/30/2023	181314
Viking Therapeutics,	VKTX	VK2809	Non-Alcoholic Steatohepatitis (NASH)	IIb	Phase IIb - VOYAGE - Top-	06/30/2023	163697
VistaGen Therapeutics,	VTGN	PH-94B	Social Anxiety Disorder (SAD)	III	Phase III PALISADE-2 -	06/30/2023	171517
Visus Therapeutics, Inc.		VTI-001	Refractive Errors (Ophthalmology)	III	Phase III BRIO-II - Top-Line Results	06/30/2023	175949
Visus Therapeutics, Inc.		VTI-001	Refractive Errors (Ophthalmology)	III	Phase III BRIO-I - Top-Line Results	06/30/2023	175950
Woolsey		Bravyl	Vascular Dementia	II	Phase IIa - FOUND - Top-	05/31/2023	167294
Woolsey		Bravyl	Progressive Supranuclear Palsy	II	Phase IIa - ROCKIT-1 -	05/31/2023	167295
X4 Pharmaceuticals,	XFOR	Mavorixafor	Primary Immunodeficiencies	III	Pre-NDA Meeting with	06/30/2023	182723
Xenikos B.V.		T-Guard	Graft vs. Host Disease (GVHD) -	III	Phase III - Safety Run-In	06/30/2023	178381
Xspray Pharma AB	XSPRAY	Dasynoc	Chronic Myelogenous Leukemia (CML)	NDA	PDUFA for NDA - First	06/30/2023	173143
Ypsomed Group		mylife YpsoPump	Diabetes Mellitus, Type I	PMA	U.S. Approval Decision	06/30/2023	145011
Zentalis	ZNTL	ZN-c3	Sarcoma	I/II	Phase II - Top-Line	06/30/2023	171495
Zevra Therapeutics,	ZVRA	Miplyffa	Niemann-Pick Disease	III	Type C Meeting with FDA	04/30/2023	175154

CITELINE
COMMERCIAL



Citeline powers a full suite of complementary business intelligence offerings to meet the evolving needs of life science professionals to accelerate the connection of treatments to patients and patients to treatments. These patient-focused solutions and services deliver and analyze data used to drive clinical, commercial and regulatory related-decisions and create real-world opportunities for growth.

Our global teams of analysts, journalists and consultants keep their fingers on the pulse of the pharmaceutical, biomedical and medtech industries, covering it all with expert insights: key diseases, clinical trials, drug R&D and approvals, market forecasts and more. For more information on one of the world's most trusted life science partners, visit **Citeline.com**