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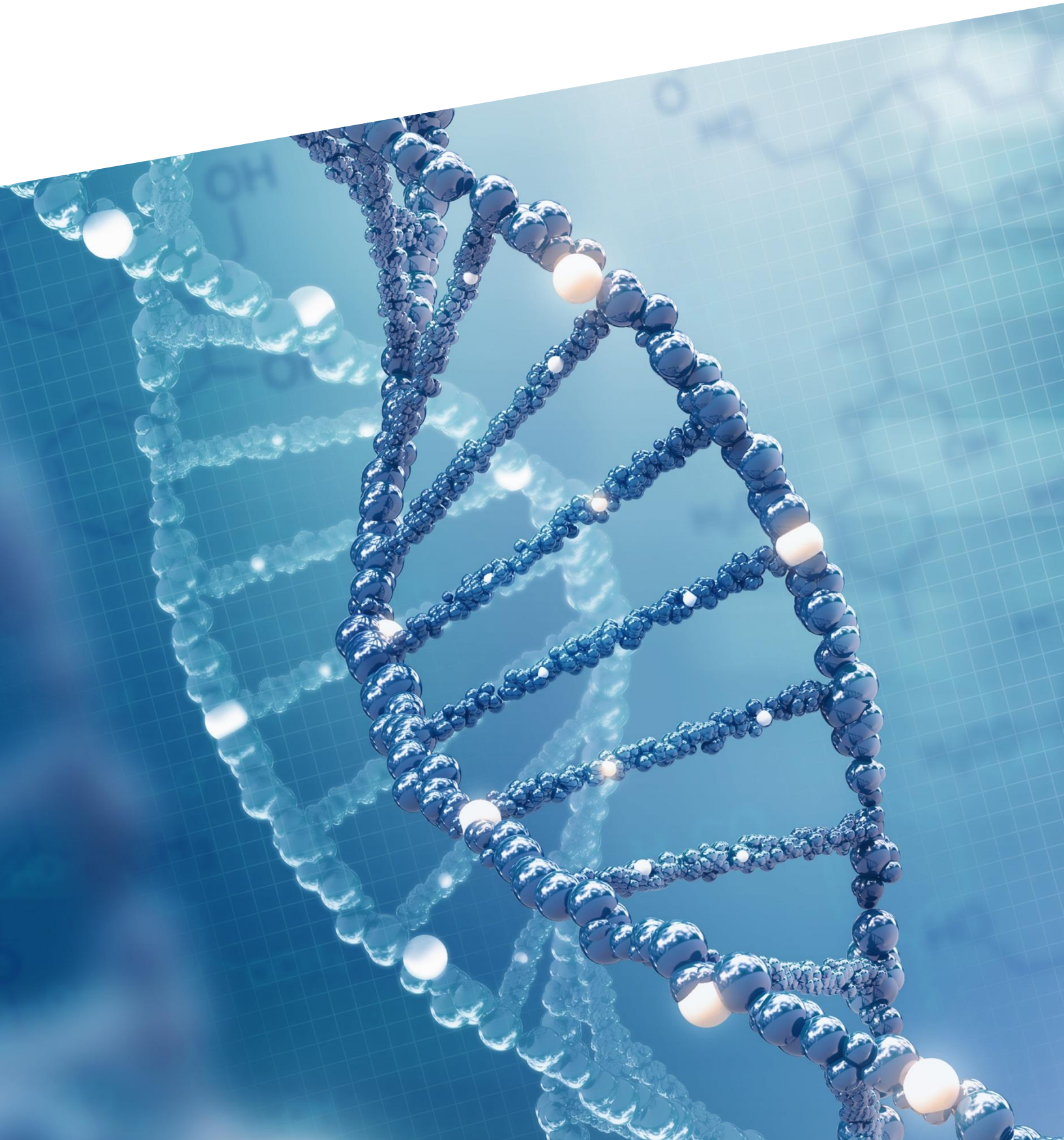
2019 JP MORGAN HEALTHCARE CONFERENCE: DAY 1



Summary

The 37th annual JP Morgan Healthcare Conference is being held in San Francisco, CA from January 7-10, 2019. A list of events and catalysts that were announced or updated at the conference today is included in this report. Below are some key points from today's company presentations.

Key Points – Day 1

Mega Cap Companies

- Merck KGaA's healthcare division update at this year's JPM conference focused primarily on the continued development of Mavenclad and Bavencio. The company highlighted that Mavenclad is now approved for multiple sclerosis in 43 countries, reimbursed in 23 markets, and is awaiting US approval in the first half of 2019. Furthermore, Merck KGaA confirmed that Bavencio's filing for first-line RCC is still on track for 2019, and that results of trials in first-line NSCLC and first-line gastric cancer are expected in late 2019. With that in mind, the company reiterated that they expect the two drugs to combine for over €2bn in sales by 2022.

  In regards to pipeline updates, top-line results of tepotinib's Phase II trial in NSCLC are expected in 2019, which could potentially allow for regulatory filings in 2020 if outcomes are positive. BTK inhibitor evobrutinib is set to proceed onto Phase III studies in multiple sclerosis in Q2 2019. Finally, in addition to the ongoing Phase II trial in first-line NSCLC, Merck KGaA will be initiating further trials for M7824 in NSCLC and biliary tract cancer in 2019.

- Novartis's (NVS) CEO Vas Narasimhan covered a lot of information in his 30-minute presentation, focusing on four main topics: re-focusing the company, driving growth / pipeline, improving productivity and margins and reshaping the company culture.

  In 2018 Novartis announced or conducted the exit of 8 subsidiaries including the tax neutral spin-off of Alcon to shareholders, which was mentioned several times. Six business were acquired in the areas of gene therapy, cell therapy and radio-drug conjugates which give an indication of the intended direction of the business. There was a renewed commitment to Sandoz and the generic side of the business, stating that Sandoz is leading in this area with 8 biosimilars on the market. Capital allocation was outlined with a commitment to invest in organic businesses, continue growing share dividends and continue buying back shares.

  In terms of sales growth, management outlined 9-month 2018 sales. Novartis's blockbuster IL-17 drug Cosentyx provided sales of $2.0bn, growing 37%. With generic competition not expected for at least 5-years, Cosentyx will continue to provide considerable revenue for Novartis for some time. Entresto, which was approved for congestive heart failure in 2015, saw growth of 117% with 9-months sales of $710m. Promacta, Tafinlar + Mekinist and Jakavi all saw growth between 27-37% with a combined 9-month revenue of $2.4bn. Novartis has a noticeably broad pipeline with a number of therapies being investigated in cardio-metabolic, neuroscience, ophthalmology and respiratory indications. Management claims to have 10+ launches planned for the next 2-years, with 6 therapies expected to have trial data readouts in 2019.

Novartis's trouble manufacturing its CAR-T cell therapy Kymriah was mentioned, with management saying the lessons learned would be useful as more cell therapies progress through the pipeline, though little detail was given.

Management recognized that they have claimed to targeting productivity and margins in the past but have provide little action in these areas. This was said to be changing with an "aggressive productivity agenda" aimed at saving $2bn by 2020 said to be underway.

Overall this was a fairly run of the mill investor presentation with few surprises. Novartis is in a solid position for 2019 with strong brands still several years from loss of exclusivity and a promising pipeline.

- Having just announced the creation of a joint venture combining **Pfizer's (PFE)** consumer healthcare division with that of GlaxoSmithKline's in December 2018, no additional transformative deals or major data releases were mentioned in Pfizer's presentation. Instead, CEO Albert Bourla used Pfizer's presentation to tout the company's expectations for strong topline growth and a substantial increase in the company's R&D spend as several of its pipeline candidates progress through late-phase development across a wide range of therapy areas. Indeed, the company noted that it has 25 potential new approvals through to 2022, with 15 of those candidates considered to possess blockbuster potential. However, Pfizer will have to endure the anticipated loss of exclusivity of Lyrica in June 2019 (which had global sales of $2.6bn in the first nine months of 2018), and thus revenue growth is likely to be weighted post 2020 once the impact of Lyrica's expiry has been absorbed.

  When asked about the highest impact events anticipated in 2019, the company highlighted the potential launches of three blockbuster drugs in 2019, including the approval of Vyndaqel (tafamadis) for transthyretin amyloid cardiomyopathy, the approval of the Bavencio (avelumab) + Inlyta (axitinb) combination for advanced renal cell carcinoma, and continued uptake/new approvals of biosimilars versions of Remicade (infliximab), Humira (adalimumab), Avastin (bevacizumab), and Rituxan (rituximab). Bourla remained tight-lipped about the potential price tag of Vyndaqel, which has shown significant reduction in the combination of all-cause mortality and frequency of cardiovascular-related hospitalizations compared to placebo in transthyretin amyloid cardiomyopathy patients, but hinted at premium pricing by noting that it would be priced according to the value it brings as an effective treatment option for a rare disease which is often fatal within 4–5 years of diagnosis. Initial sales are, however, likely to be greatly limited by very low diagnosis rates, which the company hopes to improve as awareness of new therapeutic options increases.

  Pfizer also highlighted key upcoming 2019 pivotal trial readouts for PF-04965842, an orally administered Janus kinase 1 (JAK1) inhibitor in development for atopic dermatitis, and additional pivotal data from trials of tanezumab in osteoarthritis and chronic lower back pain. While tanezumab recently met its primary efficacy endpoints in patients with severe hip/knee osteoarthritis, and could have substantial appeal as a non-addictive, non-opioid analgesic, safety concerns have been raised regarding an increased frequency of severe progressive osteoarthritis versus placebo-treated patients, and thus additional data are likely to be heavily scrutinized in 2019 to better determine its risk/benefit ratio.

Finally, Pfizer also outlined its capital deployment and M&A intentions going forward, highlighting that it would continue its share buyback program in 2019 to offset the impact of higher R&D spending on earnings per share. The company also noted that the expectations of strong revenue growth from its large and diverse pipeline has rendered large scale acquisitions to boost short-term revenue growth (along the lines of the failed Allergan merger in 2016) unnecessary, and that instead the company would focus on smaller licensing/acquisition deals to bolster its pipeline in therapy areas where it currently has expertise.

Large Cap Companies

- Allergan (AGN) highlighted four key therapeutic areas that supported the company's growth as well as major pipeline updates. The Company is currently looking for global expansion in the medical aesthetic (MA) market as its MA products represent 43% of international revenues. In the eye care therapeutic area, Allergan announced top-line results of Bimatoprost sustained-release for glaucoma showing that it reduced IOP by approximately 30% at week 12 compared to timolol. Data of this second pivotal Phase III study showed consistent results that support Bimatoprost SR as a potential treatment for glaucoma. The Company plans to submit an NDA in the second half of 2019. Additionally, a BLA submission for Abicipar in neovascular Age-related Macular Degeneration (nAMD) is expected in the first half of 2019. In the Central Nervous System (CNS) therapeutic area, Allergan is awaiting approval decision on its sNDA filing for Vraylar for the treatment of depressive episodes associated with bipolar I disorder (bipolar depression) this May. Additionally, NDA submissions for Ubrogepant in acute migraine and Rapastinel in Major Depressive Disorder (MDD) are expected in the first quarter of 2019 and the second half of 2020, respectively. Lastly, Allergan anticipates top-line results of Relamorelin for diabetic gastroparesis in the first half of 2019.

- Biogen (BIIB) officials reiterated their focus on neuroscience, due to the large opportunity as the number one cause of disability and second leading cause of death after cardiovascular reasons. They noted substantial progress in expanding their potential commercial offering beyond multiple sclerosis (MS), while still demonstrating resilience and continued investment in MS (where their products treat 35% of patients), with Vumerity in review and other transformative therapies being evaluated. MS has come down from 83% to 68% of revenues, with the addition of spinal muscular atrophy (SMA) and biosimilars, and they hope to expand their offering in the early 2020s, with a broader neuromuscular franchise and drugs in stroke, movement disorders, and alzheimer's disease, and potentially further adding ophthalmology, other neurocognitive disorders, and pain later on.

  However, when questioned in the breakout about the binary risk when final data comes out for the Alzheimer's drug aducanumab in 2020, officials acknowledged there really is not another drug large enough, anywhere, that could fill the potential miss in revenues if it fails, and they did not have multiple pipeline drugs that would either. They noted that despite the failure of a number of other antibodies targeting amyloid beta, there are substantial differences among them, and recent positive data for their partnered BAN2401 helps give them more hope for aducanumab, especially since both compounds are selective for aggregates rather than monomers. Aducanumab does have a higher rate of the side effect ARIA, but officials pointed out that could mean it is more efficacious, as well. However, they again declined to say whether there would be an interim analysis in Phase III.

Officials were also questioned about the risk for novel competitors to their successful first SMA treatment, Spinraza, such as gene therapy or oral splice modulators. They did not have a completely satisfactory answer, but did note a few factors: they were still only addressing only a minority of the treatable patients; from testimonies about the efficacy of Spinraza, they did not expect its status would easily be just displaced by gene therapy; early treatment in the NURTURE study led to near normalization; and durability could be an issue for gene therapy while Spinraza has substantial data, which could be important as physicians consider the early critical period of treatment (with the message to be sure you get it right with drugs that work in the early period of time). Nevertheless, they are themselves also working on gene therapy (where they are in active discussions with regulators on the development plan) and just before the conference noted they licensed technology for oral splice modifiers, though these will be behind other drugs.

In addition to reviewing other aspects of their pipeline, officials noted that they are planning to add an additional cohort to the Phase I study of BIIB067, their SOD1 ASO for amyotropic lateral sclerosis (ALS), which could support registration (rather than starting a separate study). While that drug only targets 1-2% of ALS patients, others in the portfolio would be appropriate for larger segments. They may also be able to file BIIB092 in progressive supranuclear palsy in H2 2019 if expected Phase II data is positive.

On the threat of an international pricing index, they said that would be difficult for the industry, but complex to implement, so they had doubts it would go through, though if it does, those who can innovate would be the survivors. They also thought the US should focus on potentially massive savings from biosimilars, where unlike Europe there have been more obstacles, which would "generate headroom for innovation." They acknowledged they were needing to increase prices at a more moderate rate, but noted they are more about gains from innovation. In terms of the potential for further deals, they noted that through 2023, they would have $42bn available, levering cash flow 2-fold.

- BioMarin (BMRN) kicked off their session with new updates for three of their pipeline candidates in 2019. The company expects a filing decision during the second half of this year, in light of the completion of enrollment in the third quarter for a Phase III valoctocogene roxaparvovec trial for severe hemophilia A vitally fulfilling accelerated approval requirements. In the first quarter of this year, a Committee on Human Medicinal Products opinion is anticipated for Palynziq, with potential approval for phenylketonuria in the second quarter. BioMarin forecasts Palynziq sales of $70-100m in 2019 with a larger proportion of EU sales expected in 2020 due to time taken for reimbursement. Additionally, vosoritide is on track with enrollment for its Phase III study in pediatric patients five years and younger with achondroplasia and the drug has been well-tolerated in early dosing.

  Although 2019 revenues were not covered, BioMarin anticipates its seven approved products to deliver $2bn in revenues in 2020. For 2018, final guidance for Kuvan was lowered slightly to $430-450m from $440-480m, and that for other products were unchanged. The company has historically built its revenues by targeting ultra-orphan disorders; however, it looks to slightly change its direction by expanding its reach to include less rare disorders. BioMarin highlighted that, thus far, it has targeted indications associated with high unmet need. This has facilitated speedy approvals and allowed its products to boast a first-to-market advantage with a lack of competitors. It further emphasized that its strategy of targeting diseases where the biology is well

understood aids in dampening the risk of failure. The company aims to build out its gene therapy portfolio and become a major player in this space with the largest in-house facility in the world to eventually manufacture valoctocogene roxaparvovec, BMN 307 (a gene therapy for phenylketonuria that has a planned IND filing in 2019), and other future products. The lucrative opportunity of this facility was touted with a $6-8bn revenue potential.

For the remainder of its presentation, BioMarin focused on four of its pipeline therapies: Palynziq, vozoritide, valoctocogene roxaparvovec, and BMN307. Palynziq was reported to have experienced a strong US launch with 252 patients reimbursed- primarily formerly naïve patients (140 patients)- and the positive trend is expected to continue in 2019 with 154 patients enrolled. Phenylketonuria represents the company's largest US patient population opportunity with >11,000 adult patients. The company went on to underline the durability and continuously improving efficacy of Palynziq over time with three-year data.

Importantly, Vozoritide is being positioned for early use in the treatment algorithm, as achondroplasia is diagnosed prior to birth and the company hopes that its drug could be used from birth. The next steps for this product include generation of Phase III data in 2019, further investigation in patients under six years, and the curation of a large natural history database to assess final adult height.

Valoctocogene roxaparvovec represents an exciting, novel prospect for hemophilia that may have the potential to address substantial unmet need in this market. The one-time gene therapy may eventually be adopted to reduce the need for surgeries, improve patients' quality of life, and save long-term costs. BioMarin plans to provide an update on three-year Phase I/II data at an upcoming medical meeting in the middle of 2019 with top-line data to be released prior to the meeting. According to the company, 49% of hemophilia A cases are severe; thus, valoctocogene roxaparvovec will initially be targeting the largest patient segment. BioMarin revealed that it later aims to expand to cover moderate patients (22% of hemophilia A patients).

- This year's conference began with a less formal "fireside" chat with the CEOs of **Bristol-Myers Squibb (BMY) and Celgene (CELG)**. The session began with an introduction to the new combined company, with BMS's CEO Giovanni Caforio emphasizing the complimentary nature of the two companies with respect to both their marketed drugs and development pipelines. For example, within oncology, sales for BMS are led by Opdivo and Yervoy, which are predominantly approved for the treatment of solid tumors. Whereas Celgene's main blockbuster therapy Revlimid is approved for hematological cancers. The acquisition was framed as two strong, complimentary companies combining to form a science and innovation focused biopharma company.

  Celgene's revenue is dominated by Revlimid which is expected to incur generic competition as soon as 2020. The implications of losing this market exclusivity on the attractiveness of Celgene were discussed with BMS management understandably saying extensive due diligence had taken place. Celgene was said to be a good acquisition for BMS even in the absence of Revlimid due to the complimentary pipeline and near-term prospects. Five of Celgene previously un-marketed therapies were identified as near-term opportunities, namely: Luspatercept, Ozanimod, Liso-cel (JCAR017), bb2121 and Fedratinib. The status of the most advanced development for each of these drugs is detailed below.

- Luspatercept: US and EU regulatory applications for transfusion-dependent, lower-risk myelodysplastic syndromes (MDS) with ring sideroblasts (RS+) and transfusion-dependent beta-thalassemia are expected in the first-half 2019.
- Ozanimod: US and EU Marketing Authorization Application (MAA) submissions in relapsing multiple sclerosis (RMS) are on-track for Q1 2019.
- Liso-cel: A US biologic license application (BLA) for relapsed/refractory DLBCL is expected in the second half of 2019.
- bb2121: Data from the KarMMa Phase II pivotal trial investigating use in relapsed/refractory multiple myeloma (RRMM) is expected in the second-half of 2019.
- Fedratinib: US FDA approval for the treatment of myelofibrosis is expected by the end of 2019 with EU MAA submission planned for the first half of 2019.

The combined revenue from these therapies was rather optimistically estimated at $12-14bn. If all of these prospects come to fruition the combined BMS / Celgene would have a very attractive portfolio.

Prior to the conference Celgene announced that financial guidance for 2018 had been met, with total revenue of $15.2bn, $9.7bn of which came from Revlimid. Guidance for 2019 targets overall revenue growth at 12%, to result in $17.0bn to $17.2bn total revenue. Revlimid sales are expected to grow by 11% to approximately $10.8bn.

The portfolios of the 2 companies do appear to have complimentary features, so it will be quite interesting to see whether the acquisition pans out as the companies have portrayed. Provided the companies can stay productive during the turbulent process of combining their operation, the next few years could be more prosperous for the new Bristol-Myers Squibb and its shareholders.

- Daiichi Sankyo's global mainstay product edoxaban (Lixiana/Savaysa) is an anticoagulant used for atrial fibrillation and venous thromboembolism. At JPM, Daiichi Sankyo explained that following market launch in Q1 FY2014, edoxaban sales have grown steadily such that in Q2 FY2018 it captured 30.7% of sales in Japan compared to 31.3% for the market leading product. Daiichi indicated that edoxaban sales are expanding mainly in Japan, EU and Asia and that in Q1 2018 edoxaban had the following share of the direct oral anticoagulant market: S. Korea, 25.1%; Japan 23.9%; Taiwan 14.3%; Belgium 13.3%; Germany 13.1%; Italy 10.5%; Spain 10.1%; UK 3.9%; and USA 0.3%. US revenues have been flat for several years due to reimbursement issues. Edoxaban sales were 77.1 billion JPY in FY2017 and are forecast to reach 111 Bn JPY in FY2018 and 120 Bn JPY in FY2020. Daiichi Sankyo prescription drug revenue from Japanese sales reached 540 Bn JPY in FY 2017 making it the biggest seller of prescription drugs in Japan for two consecutive years.

  Daichii Sankyo has two business units in the US. The first is American Regent (formerly LPI) which competes in specialty branded and generic injectables including Injactafer for iron deficient anemia in patients with non-dialysis chronic kidney disease who are intolerant or refractory to oral iron. The American Regent business reported FY2017 revenues of $951 million ($310 million for Injactafer) and is forecasting FY2019 and FY2020 revenues of $1,026 million ($372 million for Injactafer) and $1,250 million, respectively.

  The second American unit is a Daiichi Sankyo unit based in New Jersey which is forecast to have FY2018 revenues of $281 million. With the loss of exclusivity of key products, Daichii Sankyo is expected to transition from a mature primary care company to one with a differentiated portfolio

centered on three areas of oncology: an antibody-drug conjugate (ADC) franchise, an acute myeloid leukemia (AML) franchise and a breakthrough science franchise which includes drugs like pexidartinib.

At JPM, Daichii Sankyo stressed the potential of DS-8201, an anti-HER2 ADC currently enrolling third-line HER2 positive breast cancer patients in a pivotal Phase II trial (DESTINY-Breast 01). While Herceptin (trastuzumab) is approved for first-line HER2 positive patients and Kadcyla (trastuzumab emtansine, an ADC), is approved for second-line patients, third-line patients have limited options. In an ongoing Phase I study in this setting (immunohistochemistry 3+ or 2+ and ISH+), the confirmed overall response rate (for patients who received 5.4 or 6.4 mg/kg) was 59.5% (66/111 patients) and the median duration of response was 20.7 months. Interstitial lung disease (ILD) was identified as a rate-limiting toxicity and 5.4 mg/kg was selected as the pivotal trial dose based on safety, efficacy and exposure date. In 269 patients treated with this lower dose there was 1 case of Grade 5 ILD (0.4%), no cases of Grade 4 and 2 cases of Grade 3 (0.7%) for a Grade 3 or higher rate of 1.11%. During the Q&A, a company representative said that the incidence of Grade 3 or higher ILD with Tagrisso was 4%.

There are currently three ongoing Phase III trials for DS-8201: DESTINY-Breast02 (post Kadcyla vs physician's choice), DESTINY-Breast03 (vs Kadcyla) and DESTINY-Breast04 (for HER2-low patients). HER2 low patients have no approved HER2 targeted therapy and represent 43.9% of metastatic breast cancer patients (6.9% HR-/HER2-low, 37% HR+/HER2-low). The HER2-low patients enrolled in DESTINY-Breast04 include IHC2+/ISH- or IHC1+ with HR+/no prior CDK, HR+/prior CDK or HR-).

There are also plans to evaluate DS-8201 in first-line metastatic as well as early breast cancer (neo-adjuvant and adjuvant). Combinations will be investigated including with IO agents. A trial with Opdivo has initiated in breast and bladder cancer while deals have been signed with Merck for a Keytruda combination trial (breast and NSCLC) and with MERCK KGaA/Pfizer for a Bavencio combination study in patients with various solid tumors. Other combinations include with hormonal therapy, CDK4/6 inhibitors, PARP inhibitors and dual anti-HER2 combinations. DS-8201 is also being investigated in other indications. A pivotal Phase II trial (DESTINY-Gastric01) is currently evaluating DS-8201 in third-line HER2 expressing metastatic gastric cancer (vs physicians choice). Herceptin is the only approved HER2 targeted therapy for gastric cancer. A Phase III trial is also under preparation for the second-line setting (vs standard of care) and is expected to initiate in FY2019. There is an ongoing Phase II trial for HER2 expressing/mutated NCSLC and an ongoing Phase II trial for HER2 expressing/mutated CRC. The latter two indications have no approved HER2 targeted therapy. For gastric cancer, NSCLC and CRC there are plans to look at HER2 low patients and to evaluate combinations with VEGFi, chemotherapy and IO agents. There are also plans to look at early disease gastric cancer.

At JPM Daiichi Sankyo also mentioned Phase I/II data with U3-1402 an ADC specific for HER3 and which reported an ORR of 47% (15/32) and a disease control rate of 94% (30/32) at ASCO 2018. During the Q&A, a company representative discussed the AML franchise and noted that while Astella's Xospata was approved first for the salvage setting, survival data has not been released. He also explained that quizartinib is expected to report data from pivotal trials in first-line AML significantly earlier than Xospata. Daiichi Sankyo is developing IDH inhibitors and MDM2 inhibitors for use in combination with quizartinib.

Daiichi Sankyo is making significant investments in the oncology business with R&D investments expected to total 1.1 Tn JPY over 5 years with 25 Bn JPY or more in capital expenditures. Revenue from the oncology business is forecast to hit 40 Bn JPY in FY2020, 150 Bn JPY in FY2022 and 500 Bn JPY in FY2025. Total revenues for Daiichi Sankyo are forecast to hit 910 Bn JPY in FY2018, 960 Bn JPY in FY2020 and 1,100 Bn JPY in 2022.

- Dexcom (DXCM) remains bullish with their new product line, and outlined the large market potential for the G6 Continuous Glucose Monitor (CGM), which can be used across different market segments such as hospital care, assisted living, gestational diabetes, and Type II Diabetes. Dexcom reinforced the strong core technology of the G6 CGM, which when compared to labeling of their competitors devices (Abbott, Medtronic, Senseonics), the G6 is the only CGM that really requires no finger sticks, which are only suggested when the readings do match symptoms or expectations. Dexcom also provided progress updates on the Verily-Dexcom collaboration. The Verily CGM will be called the G7, and is expected to launch in later 2020 with a full roll-out in 2021. The G7 will require no collaboration, will have a 14-15 day sensor wear period, improved software and pairing, and features disposable electronics.

- As expected, this year's discussion with Gilead (GILD) management was led by conversation surrounding incoming CEO Daniel O'Day. Interim CEO and Chief Patient Officer Gregg Alton provided background into the selection and hiring of O'Day, noting that his commitment to science and innovation, his wide experience in the health care environment, and overall cultural fit gave Gilead management the confidence in O'Day to be a stable force for the company's continued forward momentum.

  When asked about points of focus for investors heading into 2019, the company outlined five key areas. First up, a return to growth, especially through top-line product revenue. While they noted a decline in HCV revenue during last year's JPM meetings, Gilead is seeing stabilization and less volatility in this space. The firm also reconfirmed its commitment to building out the cell therapy space.

  Key second and third focus points were operational excellence—continuing to have a strong presence in the HIV market in the face of competition, as the area provides an area of major long-term growth for the firm—and upcoming data readouts. In HIV, EVP of Worldwide Commercial Operations Laura Hamill reminds investors that the company has now launched 11 HIV products in the last 17 years, including most recently the entrance to market of Biktarvy (bictegravir/emtrictabine/tenofovir). She notes that Biktarvy set the record for the most successful product launch and is currently the number one product for treatment-naiive and switch patients in the US. (It was also granted Fast Track designation in France.) John McHutchison, CSO and VP of R&D, noted that investors can plan to see five separate Phase III reports in the areas of NASH (selonsertib), rheumatoid arthritis (filgotinib), and HIV. When questioned about filing timelines for filgotinib, McHutchison noted that the dates are still to be determined pending the outcoming of necessary male safety studies, but filings could come sooner in ex-US countries where those studies are not required.

  The last two points centered on building Gilead's leadership in the cell therapy space and leveraging the company's overall financial strength. In cell therapy, Yescarta was the focus, with Gilead reiterating positive results presented at the ASH meeting in December 2018, reporting strong remission rates and positive safety and efficacy data both in trial and real-world data. CFO

Robin Washington rounded up the discussion on Gilead's overall financial strength and priorities for capital allocation. She noted the theme of financial consistency for 2019, with the company remaining focused on M&A potential, core therapy areas (oncology, NASH and other liver diseases, and inflammation), dividends for shareholders, and debt repayment. In summarizing the company's path, Washington said Gilead will continue to "follow the science," and of course embrace opportunities where the science also includes potential for greater revenue.

- Incyte (INCY) discussed a busy 2019 catalyst schedule in their presentation, focusing on multiple late-stage products as they move beyond 2018's disappointing results for epacadostat and failure to gain approval for a more lucrative higher dose of Olumiant for rheumatoid arthritis. Management highlighted their long-term guidance for Jakafi, which they believe can reach $2.5-3.0bn in net revenue (they reiterated $1.37-1.4bn 2018 guidance in the US). The company also highlighted four projects that they believe can accelerate near-term growth. Data from pivotal trials expected in 2019 include ruxlitinib for steroid-refractory acute and chronic graft-versus-host disease (GVHD), and itacitinib for acute GVHD. In addition, data for a Phase II trial testing ruxolitinib cream for vitiligo is expected in 2019, with a Phase III program also expected to initiate in 2019. A NDA for pemigatinib in cholangiocarcinoma is also expected in 2019. Finally, the company discussed its long-term hopes for its oral, small molecule PD-L1 inhibitor INCB86550, whose unique mechanism of action gives it the potential for differentiation among other antibody-based PD-1/PD-L1 inhibitors.

- Jazz (JAZZ) Pharmaceuticals highlighted positive expectations for their 2018 revenue, as well as reiterated key upcoming catalysts for 2019 during their presentation. On November 6th, 2018, the company reported 2018 guidance of $1.86-1.90bn of total revenue and announced that they now expect revenue to be on the upper end of that estimate, with mid-single digit volume growth. With this strong showing, the company announced that it plans to continue to explore corporate development activities in 2019 through multiple acquisitions and partnerships.

  Adding to the company's ongoing development strategy for Defitelio for the treatment of serious disease associated with endothelial cell damage, Jazz announced plans to initiate an exploratory Phase II trial evaluating the drug for the prevention of CAR-T associated neurotoxicity in 2019, with the rationale that endothelial cell damage may play a role in this serious toxicity associated with CAR-T therapy. The company also reviewed its current development of Vyxeos in various AML settings, and announced plans to initiate two Phase III studies, one in front-line pediatric AML fit for intensive chemotherapy, and the other across adult ages in standard risk AML fit for intensive chemotherapy. Jazz also announced that it plans to initiate two studies evaluating Vyxeos in myelodysplatic syndrome, one in patients fit for chemotherapy, and the other in patients unfit for chemotherapy. Additionally, Jazz gave more detail surrounding the release of top-line data for JZP-258 for excessive daytime sleepiness and cataplexy associated with narcolepsy, announcing results could be expected in Spring of 2019.

  In terms of business development, Jazz reviewed its strategy to leverage its proprietary ComboiPlex technology, its partnership with ImmunoGen and their antibody-drug conjugate platform, as well as the Codiak collaboration announced on the Friday before the conference.

- Medtronic (MDT) reinforced its rich, diverse pipeline, which is the reason for the positive performance in the last 6 quarters. The company announced the European and Outside U.S. launch of its latest generation Continuous Glucose Monitor (CGM), the MiniMed 670G. The next

generation MiniMed, MiniMed 780G, is expected to launch by the end of fiscal year 2020, and the company announced that development of the artificial pancreas, the MiniMed 890G, is progressing. Medtronic highlighted its SugarIQ product, an AI-driven app, and noted that several next-generations of this product are expected to launch in the coming years. The company commented on its recent acquisition of Mazor Robotics, and announced the Mazor X Stealth, which combines the Mazor X Robot with the Stealth surgical navigation platform. For the cardiovascular pipeline, Medtronic announced that data from the TRYX WRAP-IT Study will be presented at the American College of Cardiology (ACC) Meeting in March 2019.

- Much of **Regeneron's (RGEN)** presentation at JPM was focused on the progress of flagship products Eylea, Dupixent, and Libtayo. Preliminary earnings results show that Eylea reached $4.07bn in net US sales in 2018, in line with expectations. Growth is expected to continue in the coming years with a potential label expansion in diabetic retinopathy without diabetic macular edema (PDUFA date May 13, 2019) and the development of a high dose formulation of Eylea. Dupixent is also poised for continued growth, with uptake in atopic dermatitis and asthma continuing to increase and several opportunities for further expansion slated for 2019, such as: potential approval in adolescent atopic dermatitis (PDUFA March 11, 2019), Phase III readout in pediatric atopic dermatitis, regulatory filing for chronic rhinosinusitis with nasal polyps (Q1 2019), and the initiation of Phase II/III trials in COPD. Finally, in 2018, PD-1 inhibitor Libtayo became the only approved treatment available for advanced cutaneous squamous cell carcinoma in the US. Approval in this indication in the EU is expected in 2019, and pivotal studies in basal cell carcinoma, NSCLC, and cervical cancer are underway.

  In regards to pipeline development, much of the company's focus was placed on the development of its immuno-oncology assets, and along with that, the restructuring of its partnership with Sanofi, announced in the morning. While the ongoing collaboration to commercialize Libtayo will continue, the remainder of the collaboration will be limited to two products for which Sanofi has the right to opt-in: REGN5458 (BCMA/CD3 bispecific antibody) and REGN4018 (MUC16/CD3 bispecific antibody). Regeneron will maintain exclusive rights to all other immuno-oncology assets, including REGN1979 (CD20/CD3 bispecific antibody), which has two potentially pivotal Phase II trials in follicular lymphoma and diffuse large B-cell lymphoma scheduled to initiate in 2019.

- At JPM, **Teva (TEVA)** highlighted that its ongoing restructuring program is on track with a net spend base reduction of $1.8bn already achieved and a further goal of $1.7bn in 2019 and $2.1bn in 2020. Teva believes it can achieve organic growth by maintaining a leadership in generics (being first to file for hundreds of products and having technology to match new products as well as effective portfolio management and selection of its most profitable existing projects). The company further mentioned it would emphasize building an internal biosimilars pipeline and related manufacturing capabilities. Approvals of Truxima (rituximab; a Rituxan biosimilar) and Herzuma (trastuzumab; a Herceptin biosimilar) late last year, as well as longer-term biosimilars, will be a large part of the total pharmaceutical market. It also plans to make investments focused on 20 in-house development projects in biologics in core therapeutic areas of CNS and respiratory versus pursuing outside M&A. Growing its TAPI and keeping OTC portfolios will also contribute to cash flow and profitability to reduce debt.

  Despite challenges from Copaxone (glatiramer acetate) generic competition in the US, for which the company has already seen and expects to see further declining revenues, it believes the US

generics market is stabilizing and pricing dynamics in the US are changing. Loss of exclusivity of ProAir will also happen in 2019. Copaxone and ProAir losses can be partially offset through steady growth of Austedo (for movement disorders and advantages of newly-launched migraine drug Ajovy (fremanezumab). EMA action on Ajovy is expected in H1 2019. In addition, new generic launches, including EpiPen are expected to generate revenues.

- Opening with a discussion detailing the company's historical strengths, Vertex (VRTX) pointed to their strategy of heavily investing operating expenses in research and development to focus on creating transformative medicines for specialty markets. The presentation started with, of course, a recap of their activity in cystic fibrosis, followed by early drug development outside of this area, and concluded by highlighting drivers of projected revenue growth.

  2018 milestones for Vertex' three approved cystic fibrosis medications included gaining label expansions for Kalydeco to treat patients down to one year and approval to use Orkambi in patients down to two years in the US and patients six years and older in Europe. Vertex emphasized positive data, including reductions in pulmonary exacerbations, hospitalizations, lung transplants, and mortality, from a long-term real-world US observational study of Kalydeco to demonstrate the importance of treating the disease as early as possible. In 2018, Vertex also launched Symdeco in the US and gained approval for the drug in the EU (as Symkevi) for patients ages 12 and up.

  Vertex expects to see Phase III data from VX-445 in the first quarter and will then select a triple combination regimen to submit an NDA by mid-2019 with the intention of getting this treatment to patients no later than 2020. This would give the company the ability to reach up to 90% of cystic fibrosis patients, including F508del/minimal function patients, with the goal of improving efficacy all the way up to carrier levels.

  Harnessing strategic lessons from their cystic fibrosis approach to drug development, the company will focus on transformative medicines with validated targets, rapid approval pathways, and using predictive assays and clinical biomarkers to move into other therapeutic areas. Among those areas specifically being explored are small molecule correctors to treat alpha-1 anti-trypsin deficiency, which have demonstrated proof-of-mechanism in preclinical studies and moved into the clinic in December 2018.

  Vertex also expects to initiate dosing in early phase gene editing trials with partner CRISPR Therapeutics. Other ex-cystic fibrosis programs that were mentioned included NaV1.8 inhibitor, VX-150, that demonstrated efficacy in three types of pain in clinical studies as well as potential candidates to treat focal segmental glomerulosclerosis.

  Wrapping up with the company's revenue growth and cash flow, Vertex noted that cystic fibrosis revenue guidance for 2018 remained at $2.9-$3bn, but out of the ~37,000 CF patients eligible for current Vertex treatment, only 18,000 were being treated. They expect to see continued uptake and reimbursement for Orkambi and Symdeko outside of the US, driven at least in part by the label expansions to treat younger patients, and the potential to treat about 68,000 patients globally if a triple combination is approved.

Mid Cap Companies

- Acceleron (XLRN) provided an overview on the development of several of its drug candidates today during its presentation. Luspatercept, the company's drug for the indications of thalassemia and myelodysplastic syndrome, is being studied in two Phase III trials named BELIEVE and MEDALIST respectively. Based on the positive results presented at the 2018 American Society of Hematology Conference, Acceleron plans to submit results from both trials for publication in 2019, and to file for marketing authorization in both the United States and Europe for both indications in the first half of 2019. Other development updates include expected data readouts in 2019 for the company's neuromuscular studies of ACE-083 for the patients with facioscapulohumeral muscular dystrophy and Charcot-Marie-Tooth Disease and of ACE-2494 in healthy volunteers.

- Alnylam (ALNY) pre-announced $11-12m Q4 2018 unaudited global revenues for Onpattro, with $1.1bn in cash still on hand. While that is not large, the drug was only approved in August 2018, so it is still too early to have a good handle on prospects, and officials said they will not be able to provide guidance yet in their Q4 2018 earnings call. The progress of the launch will of course be quite interesting to watch throughout the year. They did note that by year end 2018, there were over 200 patients in the US and EU on commercial drug, with another 350 still in the early access program (EAP). Of the 250 start forms, 48% were non-EAP in origin. Encouragingly, though the drug is approved for hATTR patients with polyneuropathy, about 35% of prescribing specialists were cardiologists and 21% other non-neurologists. While officials could not provide many details, they believe the cardiologist patients were patients with cardiomyopathy with a mixed phenotype. They also noted that at large institutions, start forms may be filled out by a designated physician. They have value based, risk sharing agreements with Harvard Pilgrim, Humana, and another top 5 payor, and are negotiating another 15, such that 90% of commercial lives will be covered.

  The company is facing likely competition from oral TTR stabilizer Vyndaqel in ATTR patients with cardiomyopathy (the drug is also approved in Europe and Japan for polyneuropathy patients), which showed a 30% reduction in mortality and 32% reduction in CV hospitalizations in its CVOT. This could impact sales for Onpattro's approved polyneuropathy indication as well, as patients with a predominant cardiomyopathy profile could be placed on Vyndaqel instead (patients ultimately develop symptoms of both polyneuropathy and cardiomyopathy). Officials did announce a placebo-controlled trial for Onpattro in cardiomyopathy patients, either wild-type or hereditary, who are TTR stabilizer naïve or progressors. However, the study will not be a CVOT (cardiovascular outcomes study), but a shorter one on functional measures, with a primary endpoint of 6MWD at 12 months. The company will be doing a full CVOT with the subcutaneous knock-down drug vutrisiran, expected to initiate late 2019, though they have not yet provided details on the design and whether that will be a direct comparison with Vyndaqel. Officials reiterated that while direct comparisons on other measures are not available, Onpattro has better efficacy data in polyneuropathy (with the implication that it may be more effective in cardiomyopathy as well), and preliminary data from the APOLLO study in polyneuropathy patients showed a 50% reduction in a composite of all-cause mortality and hospitalization, as well as an improvement in 6MWT. However, we should note that the study was too small to provide a reliable estimate, and there was actually a higher rate of CV death for Onpattro, though that could well have been due to chance. Alnylam's drug revusiran had a CVOT that was suspended for increased mortality, though officials have argued in the past that could well be drug-specific. In any case, vutrisiran will have a convenient quarterly administration schedule, which could be

attractive. Vutrisiran's polyneuropathy study should have data in the 2021-2022 time frame, per officials.

There was not a great deal of news, since the company recently had an R&D day, though they went over the remainder of their pipeline and reiterated a number of catalysts. Officials did note that they will be applying the programs and strategies used for Onpattro to the givosiran launch. They also highlighted that the Medicines Company announced today that their partnered PCSK9 inhibitor inclisiran passed its 5th Phase III DSMB review, with over 2400-patient years in the program and Phase III results expected mid-year

- Array BioPharma's (ARRY) transition into a commercial stage pharmaceutical company appears to be going smoothly, as the company reported an encouraging rollout of the Braftovi/Mektovi combination for metastatic melanoma. Importantly, the company announced that they've observed a large number of US prescribers switch their patients from the standard-of-care for BRAF-mutant melanoma, the combination of Tafinlar/Mekinist, to Braftovi/Mektovi, driven largely by results seen in the COLUMBUS trial. In this trial, Braftovi/Mektovi showed the best in-class numerical results for overall survival seen to date in BRAF-mutant patients, as well as a favorable toxicity profile.

  Going into 2019, Array emphasized the importance of the Phase III BEACON trial, evaluating the combination of Braftovi/Mektovi/cetuximab in previously-treated BRAF-mutant colorectal cancer (CRC). As the number of treatable patients in this indication exceeds the number of treatable patients in melanoma, approval for this indication is seen by the company as a larger value-driver. With enrollment for this trial completed, Array expects top-line results of an interim analysis to be release in H1 2019, and announced that they plan to seek accelerated approval in the US if results of this analysis are positive. The combination received the FDA's Breakthrough Therapy designation for CRC in August of 2018 based on results from BEACON's safety lead-in.

  Array also discussed their partnerships with Bristol-Myers Squibb, Merck, and Pfizer, testing Mektovi combinations in previously-treated, RAS-mutant, microsatellite stable (MSS) CRC, first-or second-line MSS CRC, and previously-treated pancreatic cancer/non-small cell lung cancer, respectively. Though these trials are exploratory in nature, they have the potential to drive significant value for Array.

- Ascendis (ASND) primarily gave updates for their three endocrinology rare disease products. Firstly, officials expressed their confidence in their most advanced product TransCon hGH for growth hormone deficiency (GHD) in children due to the encouraging safety profile observed in the pivotal Phase III height trial that is consistent with daily human growth hormone (hGH). In addition, Ascendis were satisfied that only one participant discontinued due to unrelated reasons and 100% of patients enrolled into the enliGHten trial. Top line data from the heiGHt trial is expected in Q1 2019, which will help bolster confidence in the platform, with database lock for filing package on track for Q3 2019. Results from the fliGHt trial and the introduction of the auto-injector to the enlighten trial are anticipated for Q2 2019. Ascendis officials were keen to note their established manufacturing and supply chain put in place early to ensure that there is no change in the product from Phase III to launch.

  Ascendis reiterated that the TransCon PTH product demonstrated positive results in the Phase I trial, which supports its use as a potential sustained replacement therapy for

hypoparathyroidism. The Phase II trial in adult patients is expected to begin in Q1 2019 and top line results are due in Q4 2019. Finally, preliminary data from the Phase I trial of TransCon CNP for achondroplasia showed a continuous exposure of CNP with one subcutaneous injection. The Phase II trial is expected to start in Q3 2019.

The company presented its new Vision 3x3 strategic Roadmap to 2025 in which the company intends to gain approval for all three endocrinology rare disease products and subsequently obtain 9 indications through a label expansion project. In addition, they aim to establish a global presence and initiate 2 more independent therapeutic areas built on TransCon technologies. They announced that oncology is their next therapeutic area as the TransCon technology is well-matched due to the two types of mechanisms: sustained systemic delivery and sustained localized delivery. Ascendis will reveal more details on the oncology pipeline during a research day on Q2 2019.

- Santen's (SZD.F) JP Morgan presentation highlighted the company's continued success in the field of ophthalmology. With revenues exceeding 200 billion yen in FY2017, Santen will to look to grow by penetrating the US, European, and Asian markets, while continuing to thrive in Japan. In the fourth quarter of 2017, Santen launched DE-117 (EYBELIS) in Japan for the treatment of glaucoma and ocular hypertension. DE-117 is an EP2 antagonist that the company plans to bring to the United States. In September 2018, Santen initiated the U.S. Phase III Spectrum III trial. Spectrum III will compare the efficacy and safety of DE-117 ophthalmic solution versus timolol maleate ophthalmic solution in patients with glaucoma or ocular hypertension. Santen will then look to launch DE-117 in the U.S. in its fiscal year 2020, provided the Spectrum III results are positive.

  Sales continue to grow for Santen as the company forecasts 237 JPY billion in sales for FY2018 (up 10% from FY2013). Santen saw rapid growth in the EMEA and Asian markets as forecasted sales for FY2018 are 39 JPY billion and 36 JPY billion respectively (up 27% and 25% from FY2013). In Japan, sales remain steady at 161 JPY billion (up 8% from FY2013). Santen will look to continue these positive trends in 2019, with the recent launches of DE-089 (Prolacria) in China (September 2018) and DE-076C (Verkazia) in the U.K. (October 2018).

- Sarepta's (SRPT) presentation emphasized the importance of their RNA and gene therapy programs and the planned path forward. The company has closed the year with $301m of net revenue ($84.4m for Q4) showing a 95% year-on-year growth rate largely due to the successful launch of Exondys 51 (eteplirsen). The President and CEO, Doug Ingram, stressed the that the strategic goal for Sarepta is going to be building out their gene therapy manufacturing capabilities, which the company is in a strong financial position to achieve, as stated during the Q&A session.

  The CEO also highlighted the progress the company made with advancing their RNA technologies beginning with the launch of Exondys 51 in 2016 for the treatment of Duchenne's Muscular Dystrophy (DMD). Sarepta has two more DMD therapies in late-stage development: golodirsen and casimersen. In December 2018, Sarepta completed its rolling NDA seeking accelerated approval for golodirsen to treat DMD patients who have genetic mutations subject to skipping exon 53 of the Duchenne gene and the estimated approval date has been set for the end of 2019 during today's presentation. For the second therapy, casimersen, following the release of biopsy results at a yet unknown point in the first quarter of 2019, filing with the FDA is expected later in the year with the target approval date of 2020.

Although there was no new clinical data presented on the micro-dystrophin program, the CEO outlined the path forward stressing the goal for the current placebo controlled trial of showing functional benefit related to expression of micro-dystrophin - one of key uncertainties related to micro-dystrophin. A target date for the commencement of a confirmatory licensure trial has been set for 2019.

- Seattle Genetics (SGEN) began its presentation by reviewing the broad impact of Adcetris regimens across a variety of settings in both Hodgkin's and non-Hodgkin's lymphoma, as well as highlighting several areas of further development for Adcentris within these two cancer types. Under the FDA's Real Time Oncology Review pilot program, the company was able to secure approval for Adcetris combined with the CHP regimen for frontline CD30-expressing peripheral T-cell lymphoma (PTCL) in less than two weeks in November of 2018. The company will continue to explore several novel chemotherapy and checkpoint inhibitor combinations with Adcetris, in disease settings including stage I/II frontline Hodgkin's lymphoma, frontline diffuse large B-cell lymphoma, second-line/salvage Hodgkin's lymphoma, and relapsed/refractory primary mediastinal B-cell lymphoma.

  Seattle Genetics also discussed its ongoing solid tumor programs, all with pivotal trials associated with them, and two of which will see data within a few months. For example, top-line results from the EV-201 trial, evaluating Enfortumab Vedotin (EV) for locally advanced or metastatic urothelial cancer, are expected in Q1 2019. Additionally, top-line results for HER2CLIMB in HER2+ metastatic breast cancer are also expected in 2019. Although more precise timing for this data release was not shared, the company did disclose that they expect enrollment to be completed by mid-2019. Additionally, the company announced that it expects enrollment for the pivotal innovaTV 204 trial, testing Tisotumab Vedotin (TV) in recurrent and/or metastatic cervical cancer, to also be completed by mid-2019.

- United Therapeutics (UTHR) provided an overview of their device and drug candidates, notably in the pulmonary hypertension indication. The Company updated timing for top-line results from the BEAT study of Beraprost 314d, which are now anticipated in the second quarter of 2019. These results will impact the approvability of the drug following failed primary and secondary endpoints of a Phase II dose response study in 2011 and subsequent reformulation of the drug in 2013 to improve efficacy and therapeutic exposure. Other notable data timing updates relate to Tyvaso, with Phase II/III INCREASE top-line results expected in 2020 and Phase III PERFECT top-line results expected the next year in 2021. Of note, the Company disclosed they filed for a label expansion of Orenitram prior to the U.S. goverment shutdown based on top-line results from the Phase III FREEDOM-EV study. The study demonstrated a statistically significant 26% reduction in the risk of a morbidity/mortality event when compared to placebo. Approval of the label expansion is anticipated in late 2019.

Small Cap Companies/Private

- Karyopharm Therapeutics (KPTI) kicked off its presentation by stating that 2019 will be an exciting year for the company and for patients with cancer. It touts itself as an industry expert in targeting nuclear export dysregulation as a mechanism to treat cancer using its SINE (Selective Inhibitor of Nuclear Export) technology. The ten-year-old firm noted that it invented and owns all its assets.

The company anticipates numerous data read-outs and potential key milestones over the upcoming 12-24 months.

Karyopharm's lead asset selinexor is an inhibitor of XPO1 and impacts tumor cells via three core mechanisms: increases nuclear levels and activation of tumor suppressor proteins, traps oncoprotein mRNA in the nucleus leading to reduced oncoprotein levels; and retains activated glucocorticoid receptor in the nucleus. The company stressed the importance of selinexor's proven activity both as an oral single agent and also when used in combination with other drugs. In 2018 it presented pivotal clinical data from the Phase IIb STORM trial in multiple myeloma (MM) and Phase IIb SADAL study in diffuse large B-cell lymphoma (DLBCL). The NDA seeking accelerated approval for selinexor for penta-refractory MM is under Priority Review by the FDA with an PDUFA action date of April 6, 2019. The MAA submission in Europe is expected in January 2019 with an EMA approval anticipated the end of the year. A second NDA of selinexor for relapsed or refractory DLBCL is expected in 2019 with a potential US launch in H1 2020.

Karyopharm also commented on the Phase III BOSTON confirmatory randomized study evaluating once-weekly selinexor and Velcade plus low-dose dexamethasone in patients with relapsed or refractory MM who have had 1-3 prior lines of therapy. Enrollment has been completed and pending progression-free survival events, top-line results from that trial could come by the end of 2019. Selinexor is also being evaluated in solid tumor malignancies. Karyopharm expects top-line data from the Phase III SEAL trial in liposarcoma by year-end 2019. The firm ended 2018 with $330m on its balance sheet.

- Mallinckrodt (MNK) opened their presentation by outlining their strategic vision- focused on treating underserved patients with severe and critical illnesses- and highlighting the breadth of their specialities, spanning a range of therapy areas, targeting patients from neonates to refractory adults, as well as covering drug-device combinations and different therapy types. The company possesses several products in late-phase development: VTS-270, terlipressin, StrataGraft, CPP-1X/sulindac, xenon gas, and stannsoporfin. These drugs plus MNK-6105 and MNK-6106 are forecast by Mallinckrodt to contribute to over 20% of their brand sales in 2023.

  As announced in 2018, the company went through plans to spin off its specialty generic business during the second half of 2019. The spinoff business will focus on complex ANDA development, as its primary growth driver, and contract manufacturing. Their Amitiza asset will follow the specialty generics company, representing a quarter of the business, and the spinoff is expected to be well capitalized. It was stated that 40% of the business would be APIs and a third, orally dosed generics. In conjunction with their Q4 2018 earnings report, to be released in late February, the company will provide additional segment guidance on the two businesses.

  Mallinckrodt have several late-phase events upcoming in 2019, including the completion of two of its lead Phase III programs, the initiation of two new Phase III programs, and developments across a myriad of indications for its main asset, H.P. Acthar Gel. The company is continuing to build out a broad evidence base for H.P. Acthar Gel, which is set to have readouts for rheumatoid arthritis and multiple sclerosis data during the first half 2019 and progress in completing enrollment for additional trials in uvelitis, lupus and ALS this year. Two Phase III trials: the CONFIRM study for terlipressin for Hepatorenal Syndrome Type 1 and the STRATA2016 trial for StrataGraft for severe burns are on track to complete during the second half of this year. If approved, these products should launch in 2020. The company further plans to initiate new

Phase III programs for MNK 6105 and stannsoporfin for hepatic encephalopathy and hyperbilirubinemia, respectively. In this way, it is attempting to revitalise stannsoporfin's approval chances after the setback of its complete response letter in August 2018.

Mallinckrodt are also working towards revamping devices for INOmax and H.P. Acthar. INOmax EVOLVE is the new nitric oxide delivery device that is significantly smaller, more portable and more user-friendly. The H.P. Acthar Gel subcutaneous self-injector being developed is intended to be the primary formulation used for all its approved indications bar infantile spasms- for which the multi-dose vial will still be relevant. Considering that H.P. Acthar Gel is adopted to treat conditions where mobility is troublesome, this updated device will be substantially more convenient for patients.

With numerous partnerships in existence, additional deals will be forged in the future as a cost-effective way to drive early science and pipeline innovation. In closing, Mallinckrodt stated that aims to generate consistent organic growth in the mid-high single digit range by developing their pipeline, continuing to operate their businesses efficiently, and adopting a disciplined capital allocation strategy. The company's strategies for 2019 will remain akin to those in the latter half of 2018, focusing on debt reduction, separating out the two businesses, cultivating their product portfolio so that no single product represents more than a third of their operating income, and enriching their pipeline within their therapeutics areas of expertise

- MyoKardia (MYOK) announced that the 6-month data for the Phase II extension trial PIONEER-OLE for obstructive hypertrophic cardiomyopathy (HCM) will be presented at the American College of Cardiology in March 2019, with 12-month data in the second half. This will be important to show longer-term effects; in addition to safety, the focus of efficacy data will be on LVOT gradient, LV ejection fraction, Nt-proBNP, and NYHA class. Positive interim 12-week data were released in October 2018, which showed that mavacamten reduced LVOT obstruction whilst maintaining ejection fraction in the normal range. This data helped set the starting dose for the Phase III EXPLORER trial. MyoKardia reiterated the enrollment timetable for EXPLORER, with topline data expected in H2 2020. They also explained that the single primary endpoint of clinical response rates will be used to capture the broad range of benefits of mavacamten. Moreover, MyoKardia highlighted the goals for the MAVERICK-HCM Phase II trial for non-obstructive HCM. Results, expected in H2 2019, will be used to identify a dosing approach for the Phase IIb trial and might provide insight into the clinical benefit of mavacmaten’s potential positive lusitropic effects.

  MyoKardia confirmed that data from the Phase IIa multiple-ascending dose study for MYK-491 in patients with stable systolic heart failure will be read out in Q4 2019. Previous results from the Phase I study showed that MYK-491 increased cardiac contractility with only mild to moderate adverse effects.

  Finally, MyoKardia reassured investors that, despite the termination of the Sanofi partnership, operations were funded into H2 2020, which will surpass the read out for EXPLORER.

- Rubius (RUBY), which went public in 2018 without any clinical stage drugs, has a quite novel approach of using allogeneic red blood cells (RBCs) to deliver drugs, focusing on enzyme replacement, immune modulation, and antigen presentation. RBCs do not have genetic material, lowering risk compared to use of other cells. While use of RBCs can limit where drugs are delivered, both that and shielding compounds within the cells can also improve safety.

In addition to reiterating the expected IND filing for RTX-134 in phenylketonuria (PKU), the company provided additional details on the PKU trial, with part 1, which will have data in H2 2019, evaluating single-dose safety, longevity of cells, and a biomarker. It is unlikely to show major shifts in phenylalanine, the key efficacy measure of the study, because there is a lot of day to day variability, with food impacting levels. The biomarker, TCA, is only produced by their enzyme, not a natural one, so it should inform them well on efficacy. Part 2 will look at reductions in phenylalanine, as well as safety and dose/schedule optimization. This clinical data will be important, as it will help establish the viability of this leg of their platform in humans. In response to a question in the breakout, officials did acknowledge that they will see in the study whether the RBCs have the same and sufficient circulating times and PK profiles. They acknowledged that studies in mice are difficult to translate as it uses a different system. However, they also said if the cells do not last long enough, that does not necessarily impact the oncology platform, as there, cells do not need to have the longevity, given they just impact the immune system. They also said, for some other diseases, like gout, they need an enzyme to pump the drug that needs to be broken down into the RBC.

For oncology, the company announced they would file an IND for RTX-240 (formerly 212; 4-1BBL co-expressed with IL-15TP) by early 2020 and announced a new oncology candidate, RTX-224 (4-1BBL co-expressed with IL-12). For RTX-240, the company highlighted preclinical data showing much greater NFkB pathway activation n T cells than for a 4-1BB agonist antibody alone, though in a mouse lung metastases model, the reduced tumor burden was only similar to (and not numerically as strong) as an antibody. However, RTX-240 did not have liver toxicity like the antibody, suggesting a better therapeutic window. In the breakout, officials noted this was important, as after initial interest in 4-1BB, toxicity emerged as a limiting factor. Also, in combination with an anti-PD-1 monoclonal antibody, RTX-240 lowered metastases more than the antibody alone. For RTX-224, the company presented new preclinical data showing that a combination with an anti-PD-1 monoclonal antibody drove deep regression in tumor growth in 9/11 mice, substantially more than the antibody alone, though only numerically slightly more than RTX-224 alone. There were signs of greater safety than recombinant IL-12 alone. There will be additional pre-clinical oncology data throughout 2019. Officials said combinations of 4-1BB with IL-15 and IL-12 have shown the best results in their surrogate preclinical systems, which is also why they decided to do combination treatment rather than 4-1BB alone.

For autoimmune therapies, they are looking to "retrain" the immune system, treating the treatment paradigm instead of chronic immunosuppressive therapy. The first clinical candidate for pemphigus vulgaris is to be selected in 2019. Officials noted that inducing tolerance has been done before, but they believe it is a matter of getting the best platform.

- SpringWorks Therapeutics started off by presenting a pipeline with several upcoming milestones planned. The company's leading program, Nirogacestat for the treatment of desmoid tumors, is anticipated to begin a Phase III trial in the first half of 2019 as well as the PD-0325901 program for NF1-associated plexiform neurofibromas is expected to also initiate a Phase IIb trial in the first half of 2019 The company reviewed the clinical data for both drugs.

  SpringWorks ended off the presentation with updates with their clinical collaborations. A Phase Ib study in combination with BioGene's Lifirafenib is expected in the first quarater of 2019, as well as two clinical collaborations, one with PD-0325901 in an undisclosed solid indication and PF-

04457875 in an undisclosed CNS indication. A clinical trial advancement decision for the two collaborations is planned to happen in mid 2019.

About the Author

Biomedtracker is an independent research service that offers proprietary clinical assessments 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 recent 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.

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Product Updates from Biomedtracker/Meddevicetracker

Acceleron Pharma, Inc. (XLRN)

Sotatercept for Pulmonary Arterial Hypertension (PAH) and Pulmonary Hypertension (PH)

Event Date:	01/07/2019
Event Type:	Trial Announcement (Clinical Analysis)
Trial Name:	Phase II - SPECTRA
Market Group:	Cardiovascular
Lead Company:	Acceleron Pharma, Inc. (XLRN)
Partner Companies:	Celgene (CELG)
Phase:	II
Change to Likelihood of Approval:	0%
Likelihood of Approval:	13% (Same As Avg.)
Average Approval:	13%

Analysis:

Acceleron announced the Phase II SPECTRA study investigating sotatercept for the treatment of pulmonary arterial hypertension (PAH). Target enrollment is 25 participants. The company plans for the study to initiate in the first quarter of 2019.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (XLRN, Slide 26)

Agios Pharmaceuticals, Inc. (AGIO)

Tibsovo for Acute Myelogenous Leukemia (AML)

Event Date:	12/31/2018
Event Type:	Regulatory - MAA Submission (Europe) (Clinical Analysis)
Trial Name:	N/A

Market Group:	Oncology
Lead Company:	Agios Pharmaceuticals, Inc. (AGIO)
Partner Companies:	CStone
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

Agios submitted an MAA for approval in Europe of Tibsovo for AML in December 2018.

Source:

Press Release 01/07/2019
J.P. Morgan Healthcare Conference 01/07/2019 (AGIO, Slide 11)

Tibsovo for Acute Myelogenous Leukemia (AML)

Event Date:	12/31/2018
Event Type:	Regulatory - sNDA/sBLA Filing (Clinical Analysis)
Trial Name:	N/A
Market Group:	Oncology
Lead Company:	Agios Pharmaceuticals, Inc. (AGIO)
Partner Companies:	CStone
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

Agios announced that it has submitted a sNDA for the approval of Tibsovo for the treatment of untreated AML in December 2018.

AG-636 for Hematologic Cancer

Event Date:	01/07/2019
Event Type:	Regulatory - IND Filing (Clinical Analysis)
Trial Name:	N/A
Market Group:	Oncology
Lead Company:	Agios Pharmaceuticals, Inc. (AGIO)
Partner Companies:	N/A
Phase:	IND
Change to Likelihood of Approval:	0%
Likelihood of Approval:	0% (Same As Avg.)
Average Approval:	N/A

Analysis:

Agio announced that the IND for AG-636 has been filed and the IND is now open.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (AGIO)

Source:

Press Release 01/07/2019
J.P. Morgan Healthcare Conference 01/07/2019 (AGIO, Slide 15)

Alnylam Pharmaceuticals, Inc. (ALNY)

Onpattro for Hereditary Transthyretin (hATTR) Amyloidosis With Polyneuropathy (Familial Amyloid Polyneuropathy)

Event Date:	01/07/2019
Event Type:	Trial Announcement (Clinical Analysis)
Trial Name:	Phase III - APOLLO B
Market Group:	Metabolic
Lead Company:	Alnylam Pharmaceuticals, Inc. (ALNY)

Partner Companies:	Sanofi (SNY) Ionis Pharmaceuticals, Inc. (IONS) Arbutus (ABUS)
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

At the 37th annual J.P. Morgan Healthcare Investor Conference, Alnylam announced the Phase III - APOLLO B study. The study is expected to initiate in mid-2019.

Phase III - APOLLO B Design

The Phase III - APOLLO B study is a randomized, double-blind, placebo-controlled study in hereditary transthyretin (hATTR) amyloidosis patients with cardiomyopathy. The primary endpoint is the change in 6 Minute Walking Distance (6-MWD) after 12 months. Secondary endpoints include cardiomyopathy symptoms or health status, death and hospitalization outcomes, and cardiac biomarkers.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (ALNY, Slide 16)

Vutrisiran for Hereditary Transthyretin (hATTR) Amyloidosis With Polyneuropathy (Familial Amyloid Polyneuropathy)

Event Date:	01/07/2019
Event Type:	Trial Announcement - Initiation (Clinical Analysis)
Trial Name:	Phase III - HELIOS A
Market Group:	Metabolic
Lead Company:	Alnylam Pharmaceuticals, Inc. (ALNY)
Partner Companies:	Sanofi (SNY)
Phase:	III
Change to Likelihood of Approval:	N/A
Likelihood of Approval:	62% (Same As Avg.)
Average Approval:	62%

Analysis:

Alnylam announced that it has initiated the Phase III - HELIOS-A study investigating vutrisiran for patients with hereditary transthyretin (hATTR) amyloidosis with polyneuropathy. Target enrollment is 160 participants. The company expects a data readout in 2021-2022.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (ALNY, Slide 18)

Array BioPharma (ARRY)

Braftovi for Melanoma

Event Date:	07/31/2018
Event Type:	Progress Update - Product Launch (U.S.) (Clinical Analysis)
Trial Name:	N/A
Market Group:	Oncology
Lead Company:	Array BioPharma, Inc. (ARRY)
Partner Companies:	Ono (4528:JP) Pierre Fabre
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

Array announced the company launched Braftovi + Mektovi for the treatment of melanoma in July 2018.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (ARRY)

Mektovi for Melanoma

Event Date:	07/31/2018
Event Type:	Progress Update - Product Launch (U.S.) (Clinical Analysis)
Trial Name:	N/A

Market Group:	Oncology
Lead Company:	Array BioPharma, Inc. (ARRY)
Partner Companies:	Ono (4528:JP) Pierre Fabre
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

Array announced the company launched Braftovi + Mektovi for the treatment of melanoma in July 2018.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (ARRY)

Mektovi for Colorectal Cancer (CRC)

Event Date:	10/15/2018
Event Type:	Trial Announcement - Initiation (Clinical Analysis)
Trial Name:	Phase II - ANCHOR-CRC
Market Group:	Oncology
Lead Company:	Array BioPharma, Inc. (ARRY)
Partner Companies:	Ono (4528:JP) Pierre Fabre
Phase:	III
Change to Likelihood of Approval:	0%
Likelihood of Approval:	35% (Same As Avg.)
Average Approval:	35%

Analysis:

Array Biopharma announced the Phase II ANCHOR-CRC study of binimetinib and encorafenib plus cetuximab was initiated earlier in October 2018. We estimate that the trial was initiated in mid-October 2018.

Source:

Sagient Analysis
J.P. Morgan Healthcare Conference 01/07/2019 (ARRY, Slide 4)
Company Q3 Earnings Slides 10/25/2018 (ARRY, Q1FY19)

Braftovi for Colorectal Cancer (CRC)

Event Date:	10/15/2018
Event Type:	Trial Announcement - Initiation (Clinical Analysis)
Trial Name:	Phase II - ANCHOR-CRC
Market Group:	Oncology
Lead Company:	Array BioPharma, Inc. (ARRY)
Partner Companies:	Ono (4528:JP) Pierre Fabre
Phase:	III
Change to Likelihood of Approval:	0%
Likelihood of Approval:	37% (2% Above Avg.)
Average Approval:	35%

Analysis:

Array Biopharma announced the Phase II ANCHOR-CRC study of binimetinib and encorafenib plus cetuximab was initiated earlier in October 2018. We estimate that the trial was initiated in mid-October 2018.

Source:

Sagient Analysis
J.P. Morgan Healthcare Conference 01/07/2019 (ARRY, Slide 4)
Company Q3 Earnings Slides 10/25/2018 (ARRY, Q1FY19)

Erbitux for Colorectal Cancer (CRC)

Event Date:	10/15/2018
Event Type:	Trial Announcement - Initiation (Clinical Analysis)
Trial Name:	N/A
Market Group:	Oncology
Lead Company:	Eli Lilly & Company (LLY)

Partner Companies:	Sanofi (SNY) Bristol-Myers Squibb (BMY) Merck KGaA (MKGAY) Yeda R&D Co.
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

Array Biopharma announced the Phase II ANCHOR-CRC study of binimetinib and encorafenib plus cetuximab was initiated earlier in October 2018. We estimate that the trial was initiated in mid-October 2018.

Source:

Sagient Analysis
J.P. Morgan Healthcare Conference 01/07/2019 (ARRY, Slide 4)
Company Q3 Earnings Slides 10/25/2018 (ARRY, Q1FY19)

Braftovi for Colorectal Cancer (CRC)

Event Date:	01/07/2019
Event Type:	Trial Announcement - Patient Enrollment Completed (Clinical Analysis)
Trial Name:	Phase III - BEACON CRC
Market Group:	Oncology
Lead Company:	Array BioPharma, Inc. (ARRY)
Partner Companies:	Ono (4528:JP) Pierre Fabre
Phase:	III
Change to Likelihood of Approval:	0%
Likelihood of Approval:	37% (2% Above Avg.)
Average Approval:	35%

Analysis:

Array Biopharma announced the company completed patient enrollment of the Phase III BEACON CRC study.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (ARRY, Slide 3, 4)

Mektovi for Colorectal Cancer (CRC)

Event Date:	01/07/2019
Event Type:	Trial Announcement - Patient Enrollment Completed (Clinical Analysis)
Trial Name:	Phase III - BEACON CRC
Market Group:	Oncology
Lead Company:	Array BioPharma, Inc. (ARRY)
Partner Companies:	Ono (4528:JP) Pierre Fabre
Phase:	III
Change to Likelihood of Approval:	0%
Likelihood of Approval:	35% (Same As Avg.)
Average Approval:	35%

Analysis:

Array Biopharma announced the company completed patient enrollment of the Phase III BEACON CRC study.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (ARRY, Slide 3, 4)

Erbitux for Colorectal Cancer (CRC)

Event Date:	01/07/2019
Event Type:	Trial Announcement - Patient Enrollment Completed (Clinical Analysis)
Trial Name:	Phase III - BEACON CRC

Market Group:	Oncology
Lead Company:	Eli Lilly & Company (LLY)
Partner Companies:	Sanofi (SNY) Bristol-Myers Squibb (BMY) Merck KGaA (MKGAY) Yeda R&D Co.
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

Array Biopharma announced the company completed patient enrollment of the Phase III BEACON CRC study.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (ARRY, Slide 3, 4)

Bausch Health Companies Inc. (BHC)

IDP-133 for Atopic Dermatitis (Eczema)

Event Date:	01/07/2019
Event Type:	Progress Update (Clinical Analysis)
Trial Name:	N/A
Market Group:	Allergy
Lead Company:	Bausch Health Companies Inc. (BHC)
Partner Companies:	N/A
Phase:	Preclinical
Change to Likelihood of Approval:	N/A
Likelihood of Approval:	0% (Same As Avg.)
Average Approval:	N/A

Analysis:

Bausch announced the development of IDP-133, a halobetasol propionate lotion, for the treatment of atopic dermatitis in its pipeline. Bausch plans to submit for approval in 2021.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (BCH, Slide 14)

BioMarin Pharmaceutical Inc. (BMRN)

Valoctocogene Roxaparvovec for Hemophilia A

Event Date:	01/07/2019
Event Type:	Trial Announcement - Trial Progressing (Clinical Analysis)
Trial Name:	Phase I/II - PoC , Phase III - GENEr8-1 (6E13 vg/kg) , Phase III - GENEr8-2 (4E13 vg/kg)
Market Group:	Hematology
Lead Company:	BioMarin Pharmaceutical Inc. (BMRN)
Partner Companies:	St. Jude UCL
Phase:	III
Change to Likelihood of Approval:	0%
Likelihood of Approval:	65% (5% Above Avg.)
Average Approval:	60%

Analysis:

BioMarin Pharmaceutical provided highlights to the investment community on its key milestones for 2019 at the 37th Annual J.P. Morgan Healthcare Conference.

During the company's presentation at the conference, BioMarin provided information on the company's development program for valoctocogene roxaparvovec gene therapy for severe hemophilia A. BioMarin has completed enrollment of the initial cohort of patients in its Phase III program intended to support a BLA submission through the accelerated approval pathway. A decision to submit a BLA through an accelerated approval pathway is tracking for the second half of 2019. If the company submits a BLA using the accelerated approval pathway, it will disclose additional information on the timing of its plans regarding the BLA submission. The complete Phase III study is targeting enrollment of 130 patients by mid-year 2019.

Valoctocogene roxaparvovec has Orphan Drug designation from the FDA and the European Medicines Agency (EMA). Valoctocogene roxaparvovec has also been accepted for Priority

Medicines (PRIME) scheme from the EMA. Additionally, the FDA has granted valoctocogene roxaparvovec Breakthrough Therapy designation.

ValRox 2019: Key Anticipated Milestones
Phase I/II data with 6e13 dose

- 3-year update "mid-year" at a key medical meeting; potential top-line data update prior to that
- Data to be included in expedited review package with goal of continued hemostasis control

Phase III with 6e13 dose

- Powered to demonstrate superiority vs. Standard of Care
- Enrollment completion (includes 6-month run-in; N=130) expected in the third quarter
- Regulatory requirements for durability for full approval will be established over the year following dosing

Accelerated Approval Pathway

- AA filing decision tracking to 2H19 and will include cohort of Phase III subjects already enrolled
- Regulatory requirements for durability for AA will be established within one year following dosing in a smaller subset of patients from Phase III cohort
- Comprehensive CMC package to be included

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (BMRN)
J.P. Morgan Healthcare Conference 01/07/2019 (BMRN, Slide 3, 16-22)

Vosoritide for Achondroplasia

Event Date:	01/07/2019
Event Type:	Trial Announcement - Trial Progressing (Clinical Analysis)
Trial Name:	Phase II - 111-206 (Age 0 to <60 Months) , Phase III - Children (Ages 5-14)
Market Group:	Endocrine
Lead Company:	BioMarin Pharmaceutical Inc. (BMRN)
Partner Companies:	Chugai Pharmaceutical (4519:JP)
Phase:	III
Change to Likelihood of Approval:	0%
Likelihood of Approval:	66% (6% Above Avg.)

Average Approval:	60%

Analysis:

BioMarin Pharmaceutical provided highlights to the investment community on its key milestones for 2019 at the 37th Annual J.P. Morgan Healthcare Conference. BioMarin provided an update on its clinical program for vosoritide, an analog of C-type Natriuretic Peptide (CNP), in children with achondroplasia, the most common form of disproportionate short stature in humans. Vosoritide has been granted orphan drug designation in both the U.S. and Europe.

BioMarin expects top line results from the fully enrolled Phase III study of vosoritide in children by year end 2019. The Phase II study in infants and young children up to age 5 with achondroplasia study enrollment is on track, and in this early part of the study, vosoritide is generally well-tolerated.

The global Phase III study is a randomized, placebo-controlled study of vosoritide in approximately 110 children with achondroplasia ages 5-14 for 52 weeks. The study will be followed by a subsequent open-label extension. Children in this study will have completed a minimum six-month baseline study to determine their respective baseline growth velocity prior to entering the Phase III study. Vosoritide is being tested in children whose growth plates are still open. This is approximately 25% of people with achondroplasia. The primary endpoint of the study is the change in growth velocity from baseline over one year in children treated compared to placebo. The company also plans to augment the growth velocity data with assessments of proportionality and functionality.

The Phase II vosoritide study is a randomized, placebo-controlled study of vosoritide in approximately 70 infants and young children with achondroplasia ages zero to less than 60 months for 52 weeks. The study will be followed by a subsequent open-label extension. Children in this study will have completed a minimum three-month baseline study to determine their respective baseline growth prior to entering the Phase II study. The primary objectives of the study are to evaluate safety, tolerability, and the effect of vosoritide on height Z-scores, which is the number of standard deviations in relation to the mean height of age-matched, average stature children. The company also plans to augment the height Z-score data with assessments including proportionality, functionality, quality of life, sleep apnea, and foramen magnum dimension, as well as the advent of major illnesses and surgeries.

Vosoritide, administered in over 28,000 injections, was generally well tolerated at all doses. The majority of adverse events (AEs) were mild and no serious adverse events (SAEs) were reported as study drug-related. Across all doses, injection site reactions and hypotension were the most common drug-related AEs. All injection site reaction events were transient. AEs of hypotension were mild, transient and resolved without medical intervention, and the majority were asymptomatic and reported in context of routine blood pressure measurements. No new safety findings were observed at the 30 µg/kg/day dose.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (BMRN)
J.P. Morgan Healthcare Conference 01/07/2019 (BMRN, Slide 3, 10-15)

Palynziq for Phenylketonuria (PKU)

Event Date:	01/07/2019
Event Type:	Regulatory - Progress Update (Clinical Analysis)

Trial Name:	N/A
Market Group:	Metabolic
Lead Company:	BioMarin Pharmaceutical Inc. (BMRN)
Partner Companies:	Merck KGaA (MKGAY)
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

BioMarin Pharmaceutical provided highlights to the investment community on its key milestones for 2019 at the 37th Annual J.P. Morgan Healthcare Conference. BioMarin noted during the presentation that the company is anticipating an opinion from the Committee for Medicinal Products for Human Use (CHMP), the scientific committee of the European Medicines Agency (EMA) on Palynziq (pegvaliase) Injection for the treatment of patients 16 and older with Phenylketonuria (PKU) in the first quarter of 2019. If the CHMP provides a positive opinion in the first quarter of 2019, then in the second quarter of 2019, it is possible that the European Commission (EC) could provide marketing authorization in the European Union. In addition, the company provided 2019 global revenue guidance for Palynziq of between $70 and $100 million.

Palynziq, a PEGylated recombinant phenylalanine ammonia lyase enzyme approved in the United States in May 2018, is the first approved enzyme substitution therapy to target the underlying cause of phenylketonuria (PKU) by helping the body to break down Phe. PKU is a rare genetic disease that manifests at birth and results in a variety of cumulative toxic effects on the brain. Palynziq is BioMarin's second approved treatment for this serious condition. In March 2018, the European Medicines Agency accepted BioMarin's submission of a Marketing Authorization Application for Palynziq.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (BMRN)
J.P. Morgan Healthcare Conference 01/07/2019 (BMRN, Slide 3, 6-9)

Celgene Corporation (CELG)

Fedratinib for Myelofibrosis (MF)

Event Date:	12/31/2018
Event Type:	Regulatory - NDA/BLA Filing (Clinical Analysis)
Trial Name:	N/A

Market Group:	Oncology
Lead Company:	Celgene Corporation (CELG)
Partner Companies:	N/A
Phase:	NDA/BLA
Change to Likelihood of Approval:	47%
Likelihood of Approval:	82% (Same As Avg.)
Average Approval:	82%

Analysis:

Celgene announced that the U.S. FDA approval of fedratinib is expected by year-end 2019. The company confirmed that the fedratinib NDA was submitted. We suspect that the submission occurred in December 2018.

Source:

Sagient Analysis
J.P. Morgan Healthcare Conference 01/07/2019 (CELG)

Revlimid for Indolent Non-Hodgkin's Lymphoma (Including Follicular Lymphoma) - NHL

Event Date:	12/31/2018
Event Type:	Regulatory - sNDA/sBLA Filing (Clinical Analysis)
Trial Name:	N/A
Market Group:	Oncology
Lead Company:	Celgene Corporation (CELG)
Partner Companies:	BeiGene (BGNE)
Phase:	NDA/BLA
Change to Likelihood of Approval:	47%
Likelihood of Approval:	93% (11% Above Avg.)
Average Approval:	82%

Analysis:

Celgene announced that approval is expected by the U.S. Food and Drug Administration (FDA) on the supplemental New Drug Application (sNDA) for REVLIMID in combination with rituximab in relapsed/refractory indolent lymphoma (AUGMENT). The company confirmed that the REVLIMID sNDA was submitted. We suspect that the submission occurred in December 2018.

Source:

Sagient Analysis
J.P. Morgan Healthcare Conference 01/07/2019 (CELG)

Otezla for Behçet Syndrome

Event Date:	01/07/2019
Event Type:	Regulatory - Supplemental Filing (Japan) (Clinical Analysis)
Trial Name:	N/A
Market Group:	Autoimmune/immunology
Lead Company:	Celgene Corporation (CELG)
Partner Companies:	N/A
Phase:	NDA/BLA
Change to Likelihood of Approval:	0%
Likelihood of Approval:	92% (5% Above Avg.)
Average Approval:	87%

Analysis:

Approval is expected by the U.S. FDA for the OTEZLA sNDA in Behçet's disease with a Prescription Drug User Fee Act (PDUFA) action date of July 21, 2019. Approval by the PMDA in Japan is expected in the second half of 2019.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (CELG)

Revlimid for Multiple Myeloma (MM)

Event Date:	01/07/2019
Event Type:	Regulatory - Supplemental Filing (Europe) (Clinical Analysis)
Trial Name:	N/A

Market Group:	Oncology
Lead Company:	Celgene Corporation (CELG)
Partner Companies:	BeiGene (BGNE)
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

Approval is expected by the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) for REVLIMID in combination with bortezomib and dexamethasone (RVd) in newly diagnosed multiple myeloma (NDMM) in 2019.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (CELG)

Pomalyst for Multiple Myeloma (MM)

Event Date:	01/07/2019
Event Type:	Regulatory - Supplemental Filing (Japan) (Clinical Analysis)
Trial Name:	N/A
Market Group:	Oncology
Lead Company:	Celgene Corporation (CELG)
Partner Companies:	N/A
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

Approval is expected by the EMA CHMP and Japan Pharmaceuticals and Medical Devices Agency (PMDA) for POMALYST/IMNOVID in combination with bortezomib and dexamethasone (PVd) in relapsed/refractory multiple myeloma (RRMM) in 2019.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (CELG)

Daiichi Sankyo Co., Ltd. (DSKYF)

Savaysa for Venous Thromboembolism (VTE)

Event Date:	08/31/2018
Event Type:	Progress Update - Product Launch (Emerging Markets) (Clinical Analysis)
Trial Name:	N/A
Market Group:	Hematology
Lead Company:	Daiichi Sankyo Co., Ltd. (DSKYF)
Partner Companies:	Merck (MRK) Servier Daewoong (069620:KS)
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

Daiichi Sankyo announced edoxaban was launched in Brazil in August 2018.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (DSKYF, Slide 10)

Savaysa for Stroke Prevention in Atrial Fibrillation (SPAF)

Event Date:	08/31/2018
Event Type:	Progress Update - Product Launch (Emerging Markets) (Clinical Analysis)
Trial Name:	N/A
Market Group:	Hematology

Lead Company:	Daiichi Sankyo Co., Ltd. (DSKYF)
Partner Companies:	Merck (MRK) Servier Daewoong (069620:KS)
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

Daiichi Sankyo announced edoxaban was launched in Brazil in August 2018.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (DSKYF, Slide 10)

DS-8201 for Breast Cancer

Event Date:	12/12/2018
Event Type:	Trial Data - Updated Results (Clinical Analysis)
Trial Name:	Phase I - Solid Tumors
Market Group:	Oncology
Lead Company:	Daiichi Sankyo Co., Ltd. (DSKYF)
Partner Companies:	N/A
Phase:	III
Change to Likelihood of Approval:	0%
Likelihood of Approval:	37% (2% Above Avg.)
Average Approval:	35%

	Treatment
Treatment Description	DS-8201
Number of Patients	N/A
Number of Evaluable Patients	111
Confirmed Overall Response Rate	59.500 %

Median Duration of Response	20.700 Months

Analysis:

Daiichi Sankyo announced new data from its Phase I trial of DS-8201 in HER2 positive breast cancer.

Data from this trial were seen earlier in December 2018.

Design
Subjects analyzed received 5.4 or 6.4 mg/kg of DS-8201 with ≥2 postbaseline scans, or who had progressive disease or discontinued treatment for any reason before their second postbaseline scan.

Results
Results can be seen in the table above.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (DSKYF, Slide 27)
Company R&D/Analyst Day Slides 12/12/2018 (DSKYF, Slide 27)

DS-8201 for Breast Cancer

Event Date:	12/19/2018
Event Type:	Trial Announcement - Initiation (Clinical Analysis)
Trial Name:	Phase III - DESTINY-Breast04 (HER2-low)
Market Group:	Oncology
Lead Company:	Daiichi Sankyo Co., Ltd. (DSKYF)
Partner Companies:	N/A
Phase:	III
Change to Likelihood of Approval:	0%
Likelihood of Approval:	37% (2% Above Avg.)
Average Approval:	35%

Analysis:

Daiichi Sankyo announced a Phase III, multicenter, randomized, open-label, active controlled trial of DS-8201a, an anti-HER2-antibody drug conjugate (ADC), versus treatment of physician's choice for HER2-low, unresectable and/or metastatic breast cancer subjects is currently recruiting participants. Estimated enrollment is 540 patients.

Source:

www.clinicaltrials.gov
J.P. Morgan Healthcare Conference 01/07/2019 (DSKYF, Slide 25)

DS-7300 for Solid Tumors

Event Date:	01/07/2019
Event Type:	Progress Update (Clinical Analysis)
Trial Name:	N/A
Market Group:	Oncology
Lead Company:	Daiichi Sankyo Co., Ltd. (DSKYF)
Partner Companies:	N/A
Phase:	Development Outside U.S.
Change to Likelihood of Approval:	N/A
Likelihood of Approval:	0% (Same As Avg.)
Average Approval:	N/A

Analysis:

Daiichi Sankyo lists DS-7300 in preclinical development for the treatment of solid tumors in its pipeline.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (DSKYF, Slide 24)

DS-6157 for Gastrointestinal Stromal Tumor (GIST)

Event Date:	01/07/2019
Event Type:	Progress Update (Clinical Analysis)
Trial Name:	N/A
Market Group:	Oncology
Lead Company:	Daiichi Sankyo Co., Ltd. (DSKYF)
Partner Companies:	N/A
Phase:	Development Outside U.S.
Change to Likelihood of Approval:	N/A

Likelihood of Approval:	0% (Same As Avg.)
Average Approval:	N/A

Analysis:

Daiichi Sankyo lists DS-6157 in preclinical development for the treatment of gastrointestinal stromal tumors in its pipeline.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (DSKYF, Slide 24)

DexCom, Inc. (DXCM)

Dexcom G7 Continuous Glucose Monitoring System for Diabetes Mellitus, Type I

Event Date:	01/07/2019
Event Type:	Progress Update (Clinical Analysis)
Trial Name:	N/A
Market Group:	Endocrine
Lead Company:	DexCom, Inc. (DXCM)
Partner Companies:	Alphabet (GOOGL)
Phase:	IDE
Change to Likelihood of Approval:	0%
Likelihood of Approval:	0% (Same As Avg.)
Average Approval:	N/A

Analysis:

Dexcom announced new developments to the Verily Continuous Glucose Monitor (CGM) at the 2019 J.P. Morgan Healthcare Conference. The Verily CGM will be marketed at the G7 CGM, and will provide real-time CGM, a 14-15 day sensor wear time, and disposable electronics.

The company expects to initially launch the G7 in late 2020 with a full roll out in 2021.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (DXCM)

Edwards Lifesciences Corp. (EW)

SAPIEN 3 for Cardiac Valve Surgery

Event Date:	01/07/2019
Event Type:	Progress Update - Product Launch (U.S.) (Clinical Analysis)
Trial Name:	N/A
Market Group:	Cardiovascular
Lead Company:	Edwards Lifesciences Corp. (EW)
Partner Companies:	N/A
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

Edwards announced that it has launched the Sapien 3 Ultra System in the United States and Europe.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (EW)

SAPIEN 3 for Cardiac Valve Surgery

Event Date:	01/07/2019
Event Type:	Progress Update - Product Launch (Europe) (Clinical Analysis)
Trial Name:	N/A
Market Group:	Cardiovascular
Lead Company:	Edwards Lifesciences Corp. (EW)
Partner Companies:	N/A
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

Edwards announced that it has launched the Sapien 3 Ultra System in the United States and Europe.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (EW)

Endo International plc (ENDP)

Xiaflex for Cellulite

Event Date:	01/07/2019
Event Type:	Partnership - Acquisition Announcement (Clinical Analysis)
Trial Name:	N/A
Market Group:	Dermatology
Lead Company:	Endo International plc (ENDP)
Partner Companies:	Johnson & Johnson (JNJ) Swedish Orphan Biovitrum (SOBI:SS) Asahi Kasei Corporation (AHKSY) BioSpecifics Technologies (BSTC)
Phase:	III
Change to Likelihood of Approval:	0%
Likelihood of Approval:	66% (4% Above Avg.)
Average Approval:	62%

Analysis:

Endo announces that it will expand its U.S. branded sterile injectible capabilities through the acquisition of Somerset. The acquisition is planned to close in Q1 2019.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (ENDP, Slide 7)

Frequency Therapeutics, Inc.

FX-322 for Hearing Loss - General

Event Date:	01/07/2019
Event Type:	Corporate - New Stock/Convertible Offering (Clinical Analysis)
Trial Name:	N/A

Market Group:	ENT/Dental
Lead Company:	Frequency Therapeutics, Inc.
Partner Companies:	N/A
Phase:	II
Change to Likelihood of Approval:	0%
Likelihood of Approval:	24% (Same As Avg.)
Average Approval:	24%

Analysis:

Frequency Therapeutics announced the closing of a $42 million Series B financing, which brings the total capital raised by the Company to $87 million.

The financing was led by Taiwania Capital Management, a venture capital firm with $350 million biotechnology and IT funds, and Axil Capital, a healthcare focused venture fund spun out of Mizuho Securities. Yonjin Capital and DF Investments were also new investors in the Series B financing. Existing investors Polaris Founders Capital, Alexandria Venture Investments, CoBro Ventures, Korea Investment Partners and Emigrant Capital also participated in the Series B financing.

Proceeds from the financing will support the advancement of the Company's clinical candidate, FX-322, for hearing regeneration. Top-line results from an ongoing Phase I/II study are expected in the first half of 2019. The financing will also support the continued expansion of Frequency's pipeline with new therapeutic applications from the Company's PCA Regeneration platform.

Source:

Press Release 01/07/2019
J.P. Morgan Healthcare Conference 01/07/2019 (SpringWorks, Slide 20)

Incyte Corporation (INCY)

INCB54828 for Biliary Tract Cancer

Event Date:	12/14/2018
Event Type:	Trial Announcement - Initiation (Clinical Analysis)
Trial Name:	Phase III - vs. Gemcitabine and Cisplatin (FIGHT-302)
Market Group:	Oncology
Lead Company:	Incyte Corporation (INCY)
Partner Companies:	Innovent Biologics

Phase:	III
Change to Likelihood of Approval:	25%
Likelihood of Approval:	35% (Same As Avg.)
Average Approval:	35%

Analysis:

INCY announced The FIGHT-302, Phase III, open-label, randomized, active-controlled, multicenter study to evaluate the efficacy and safety of pemigatinib versus gemcitabine plus cisplatin chemotherapy in first-line treatment of participants with unresectable or metastatic cholangiocarcinoma with FGFR2 rearrangement is currently recruiting participants. Estimated enrollment is 432 patients.

Source:

www.clinicaltrials.gov
J.P. Morgan Healthcare Conference 01/07/2019 (INCY, Slide 12)

INCB-086550 for Solid Tumors

Event Date:	01/02/2019
Event Type:	Trial Announcement - Initiation (Clinical Analysis)
Trial Name:	Phase I - First-in-Human
Market Group:	Oncology
Lead Company:	Incyte Corporation (INCY)
Partner Companies:	N/A
Phase:	I
Change to Likelihood of Approval:	N/A
Likelihood of Approval:	6% (Same As Avg.)
Average Approval:	6%

Analysis:

Incyte announced a Phase I study exploring the safety, tolerability, pharmacokinetics, and pharmacodynamics of INCB086550 in participants with advanced solid tumors is currently recruiting participants. Estimated enrollment is 57 patients.

Source:

www.clinicaltrials.gov
J.P. Morgan Healthcare Conference 01/07/2019 (INCY, Slide 22)

Ionis Pharmaceuticals, Inc. (IONS)

HTT-ASO for Huntington's Disease

Event Date:	01/07/2019
Event Type:	Trial Announcement - Initiation (Clinical Analysis)
Trial Name:	Phase III - BN40423
Market Group:	Neurology
Lead Company:	Roche Holding AG (RHHBY)
Partner Companies:	Ionis Pharmaceuticals, Inc. (IONS)
Phase:	III
Change to Likelihood of Approval:	35%
Likelihood of Approval:	54% (2% Above Avg.)
Average Approval:	52%

Analysis:

Ionis announced the Phase III trial of ISIS HTTRx in Huntington's Disease is underway at the JP Morgan Healthcare Conference.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (IONS)

AKCEA-TTR-LRx for Hereditary Transthyretin (hATTR) Amyloidosis With Polyneuropathy (Familial Amyloid Polyneuropathy)

Event Date:	01/07/2019
Event Type:	Trial Announcement - Initiation (Clinical Analysis)
Trial Name:	Phase I/II - ION-682884-CS1
Market Group:	Metabolic
Lead Company:	Akcea Therapeutics, Inc. (AKCA)
Partner Companies:	Ionis Pharmaceuticals, Inc. (IONS)
Phase:	II
Change to Likelihood of Approval:	24%
Likelihood of Approval:	24% (Same As Avg.)

Average Approval:	24%

Analysis:

Ionis announced the Phase I/II trial of AKCEA-TTR-LRx in Hereditary Transthyretin is underway.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (IONS)

Jazz Pharmaceuticals plc (JAZZ)

Defitelio for Drug Toxicity

Event Date:	01/07/2019
Event Type:	Trial Announcement (Clinical Analysis)
Trial Name:	N/A
Market Group:	Not Specified
Lead Company:	Jazz Pharmaceuticals plc (JAZZ)
Partner Companies:	Alfasigma Nippon Shinyaku (4516:JP) Swedish Orphan Biovitrum (SOBI:SS) Bausch Health Companies (BHC) Medison Crinos Biologix Gen Ilac
Phase:	Preclinical
Change to Likelihood of Approval:	N/A
Likelihood of Approval:	0% (Same As Avg.)
Average Approval:	N/A

Analysis:

Jazz Pharmaceuticals plans to initiate an exploratory Phase II study of Defitelio in 2019 for the prevention of CAR-T associated neurotoxicity in approximately 35 patients.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (JAZZ, Slide 9-10)

Kala Pharmaceuticals, Inc. (KALA)

Inveltys for Ocular Pain and/or Inflammation (Ophthalmology)

Event Date:	01/07/2019
Event Type:	Progress Update - Product Launch (U.S.) (Clinical Analysis)
Trial Name:	N/A
Market Group:	Ophthalmology
Lead Company:	Kala Pharmaceuticals, Inc. (KALA)
Partner Companies:	N/A
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

Kala announced the launch of INVELTYS (loteprednol etabonate ophthalmic suspension) 1% for the treatment of post-operative inflammation and pain following ocular surgery and the hiring of the specialty ophthalmology sales organization. INVELTYS is now in national and regional United States (U.S.) pharmaceutical distribution centers, and patients have access to INVELTYS through their local retail pharmacies.

INVELTYS was approved in August 2018 as the first and only twice-daily ocular corticosteroid indicated for the treatment of post-operative inflammation and pain following ocular surgery. INVELTYS utilizes Kala's proprietary AMPPLIFY Drug Delivery Technology to enhance penetration into target tissues of the eye. The AMPPLIFY technology also underpins KPI-121 0.25%, a product candidate for the temporary relief of signs and symptoms of dry eye disease utilizing a two-week course of therapy. Kala filed a New Drug Application (NDA) for KPI-121 0.25% with the FDA and has been granted a PDUFA target action date of August 15, 2019. Based upon the FDA's recommendation, Kala also initiated an additional Phase III clinical trial in July 2018, STRIDE 3 (STRIDE - Short Term Relief In Dry Eye), evaluating KPI-121 0.25% for the temporary relief of the signs and symptoms of dry eye disease. The Company expects to report top-line results for STRIDE 3 in the fourth quarter of 2019.

Source:

Press Release 01/07/2019 (KALA)
J.P. Morgan Healthcare Conference 01/07/2019 (KALA)

Medtronic plc (MDT)

MiniMed 670G for Diabetes Mellitus, Type I

Event Date:	01/07/2019
Event Type:	Progress Update - Product Launch (Europe) (Clinical Analysis)
Trial Name:	N/A
Market Group:	Endocrine
Lead Company:	Medtronic plc (MDT)
Partner Companies:	N/A
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

Medtronic announced that it has launched the MiniMed 670G in Europe and other non-U.S. markets.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (MDT)

Regeneron Pharmaceuticals, Inc. (REGN)

REGN5458 for Multiple Myeloma (MM)

Event Date:	01/07/2019
Event Type:	Trial Announcement - Initiation (Clinical Analysis)
Trial Name:	Phase I/II - First-In-Human
Market Group:	Oncology
Lead Company:	Regeneron Pharmaceuticals, Inc. (REGN)
Partner Companies:	N/A
Phase:	II
Change to Likelihood of Approval:	10%

Likelihood of Approval:	10% (Same As Avg.)
Average Approval:	10%

Analysis:

Regeneron announced that REGN5458 has entered clinical development and a Phase I study is already initiated.

Source:

Press Release 01/07/2019 (REGN)
J.P. Morgan Healthcare Conference 01/07/2019 (REGN, Slide 4)

Roivant Sciences, Inc.

Tapinarof for Psoriasis

Event Date:	01/07/2019
Event Type:	Trial Data - Updated Results (Clinical Analysis)
Trial Name:	Phase IIb - Study 203120
Market Group:	Autoimmune/immunology
Lead Company:	Roivant Sciences, Inc.
Partner Companies:	GlaxoSmithKline (GSK) NovaQuest Welichem Biotech (WBI:CN) Celestial Pharmaceuticals
Phase:	II
Change to Likelihood of Approval:	0%
Likelihood of Approval:	20% (Same As Avg.)
Average Approval:	20%

	Placebo	Placebo	Treatment	Treatment	Treatment	Treatment
Treatment Description	Vehicle QD mITT	Vehicle BID mITT	Tapinarof 0.5% QD mITT	Tapinarof 0.5% BID mITT	Tapinarof 1% QD mITT	Tapinarof 1% BID mITT

Number of Patients	N/A	N/A	N/A	N/A	N/A	N/A
Number of Evaluable Patients	N/A	N/A	N/A	N/A	N/A	N/A
Patients w/PGA Score of Clear (0) or Almost Clear (1) and Minimum 2-Grade Improvement in PGA Score **Baseline to Week 12** (Endpoint=Primary)	5.000 %	11.000 %	36.000 % (P< 0.0500)	46.000 % (P< 0.0500)	56.000 % (P< 0.0500)	65.000 % (P< 0.0500)
Patients w/PGA Score of Clear (0) or Almost Clear (1) and Minimum 2-Grade Improvement in PGA Score **Follow-Up to Week 16**	0.000 %	5.000 %	38.000 %	35.000 %	54.000 %	58.000 %

Analysis:

Roivant Sciences announced updated Phase IIb psoriasis results for Tapinarof at the 2019 J.P. Morgan Healthcare Conference. Tapinarof is an investigational therapeutic aryl hydrocarbon receptor modulating agent (TAMA).

Results from this study were last seen in July 2018.

Results

Tapinarof demonstrated clinically meaningful, dose-dependent improvements over vehicle on the primary endpoint of the study, the proportion of treated patients who achieved Physician Global Assessment (PGA) scores of clear or almost clear (0 or 1) after 12 weeks of treatment with a minimum of 2-grade improvement in 5-point PGA score from baseline.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (Roivant, Slide 33)

Rubius Therapeutics (RUBY)

RTX-240 for Solid Tumors

Event Date:	01/07/2019
Event Type:	Progress Update (Clinical Analysis)
Trial Name:	N/A
Market Group:	Oncology
Lead Company:	Rubius Therapeutics (RUBY)
Partner Companies:	N/A
Phase:	Preclinical
Change to Likelihood of Approval:	N/A
Likelihood of Approval:	0% (Same As Avg.)
Average Approval:	N/A

Analysis:

RTX-240 is listed in IND enabling status on the company's pipeline.

Source:

Company Website (RUBY, Pipeline)
J.P. Morgan Healthcare Conference 01/07/2019 (RUBY, Silde 8)

RTX-224 for Solid Tumors

Event Date:	01/07/2019
Event Type:	Progress Update (Clinical Analysis)
Trial Name:	N/A
Market Group:	Oncology
Lead Company:	Rubius Therapeutics (RUBY)
Partner Companies:	N/A
Phase:	Preclinical
Change to Likelihood of Approval:	N/A
Likelihood of Approval:	0% (Same As Avg.)
Average Approval:	N/A

Analysis:

Rubius announced RTX-224 as the company's new candidate for cancer.

Source:

Press Release 01/07/2019
J.P. Morgan Healthcare Conference 01/07/2019 (RUBY, Slide 19)

Santen Pharmaceutical Co., Ltd. (4536:JP)

DE-117 for Glaucoma / Ocular Hypertension (Ophthalmology)

Event Date:	09/13/2018
Event Type:	Trial Announcement - Initiation (Clinical Analysis)
Trial Name:	Phase III - Spectrum III
Market Group:	Ophthalmology
Lead Company:	Santen Pharmaceutical Co., Ltd. (4536:JP)
Partner Companies:	Ube Industries (4208:JP)
Phase:	III
Change to Likelihood of Approval:	27%
Likelihood of Approval:	51% (Same As Avg.)
Average Approval:	51%

Analysis:

The Spectrum III, Phase III, randomized, double-masked, active-controlled, parallel-group, multi-center study assessing the efficacy and safety of DE-117 ophthalmic solution compared with timolol maleate ophthalmic solution 0.5% in subjects with glaucoma or ocular hypertension is currently recruiting participants. Estimated enrollment is 430 patients.

Source:

www.clinicaltrials.gov
J.P. Morgan Healthcare Conference 01/07/2019 (Santen, Slide 19)

Verkazia for Allergic Conjunctivitis (Ophthalmology)

Event Date:	10/31/2018

Event Type:	Progress Update - Product Launch (Europe) - Individual Country (Clinical Analysis)
Trial Name:	N/A
Market Group:	Ophthalmology
Lead Company:	Santen Pharmaceutical Co., Ltd. (4536:JP)
Partner Companies:	N/A
Phase:	Development Outside U.S.
Change to Likelihood of Approval:	0%
Likelihood of Approval:	0% (Same As Avg.)
Average Approval:	N/A

Analysis:

Santen announced DE-076C was launched in the U.K. for the treatment of vernal keratoconjunctivitis in October 2018.

Source:

Pipeline Update 11/07/2018 (Santen)
J.P. Morgan Healthcare Conference 01/07/2019 (Santen, Slide 25)

Verkazia for Allergic Conjunctivitis (Ophthalmology)

Event Date:	12/21/2018
Event Type:	Regulatory - Approval (Canada) (Clinical Analysis)
Trial Name:	N/A
Market Group:	Ophthalmology
Lead Company:	Santen Pharmaceutical Co., Ltd. (4536:JP)
Partner Companies:	N/A
Phase:	Development Outside U.S.
Change to Likelihood of Approval:	0%
Likelihood of Approval:	0% (Same As Avg.)
Average Approval:	N/A

Analysis:

On December 21, 2018, Health Canada approved Verkazia for the treatment of severe vernal keratoconjunctivitis in children from 4 years of age through adolescence. Santen expects to launch Verkazia in Canada in the company's fiscal year 2018 or 2019.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (Santen, Slide 25)
www.hc-sc.gc.ca (Health Canada) 12/21/2018 (Product Monograph)

Prolacria for Dry Eye (Ophthalmology)

Event Date:	09/30/2018
Event Type:	Progress Update - Product Launch (Emerging Markets) (Clinical Analysis)
Trial Name:	N/A
Market Group:	Ophthalmology
Lead Company:	Merck & Co., Inc. (MRK)
Partner Companies:	Kissei (4547:JP) Allergan (AGN) Santen Pharmaceutical Co., Ltd. (4536:JP)
Phase:	Development Outside U.S.
Change to Likelihood of Approval:	0%
Likelihood of Approval:	0% (Same As Avg.)
Average Approval:	N/A

Analysis:

Santen announced DE-089 was launched in China in September 2018.

Source:

Pipeline Update 11/07/2018 (Santen)
J.P. Morgan Healthcare Conference 01/07/2019 (Santen, Slide 25)

Sarepta Therapeutics, Inc. (SRPT)

Microdystrophin Gene Therapy Program (NCH) for Duchenne Muscular Dystrophy (DMD)

Event Date:	12/31/2018
Event Type:	Regulatory - Meeting with FDA (Clinical Analysis)
Trial Name:	N/A
Market Group:	Metabolic
Lead Company:	Sarepta Therapeutics, Inc. (SRPT)
Partner Companies:	N/A
Phase:	II
Change to Likelihood of Approval:	0%
Likelihood of Approval:	28% (4% Above Avg.)
Average Approval:	24%

Analysis:

Sarepta announced that it met with the FDA to discuss its Micro-Dystrophin Gene Therapy Program in the fourth quarter of 2018. The FDA noted it will require to have clinical data using a commercial version for approval. Sarepta plans to conduct a confirmatory trial of its micro-dystrophin program with commercial material in 2019.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (SRPT, Slide 12)

Spark Therapeutics, Inc. (ONCE)

SPK-8011 for Hemophilia A

Event Date:	01/07/2019
Event Type:	Trial Announcement - Initiation (Clinical Analysis)
Trial Name:	N/A
Market Group:	Hematology
Lead Company:	Spark Therapeutics, Inc. (ONCE)
Partner Companies:	N/A
Phase:	III
Change to Likelihood of Approval:	30%

Likelihood of Approval:	58% (2% Below Avg.)
Average Approval:	60%

Analysis:

Spark Therapeutics reported that the Phase III run-in trial of SPK-8011 in hemophilia A has been initiated.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (ONCE, Slides 3, 18)

SpringWorks Therapeutics, LLC

PD-0325901 for Neurofibromatosis (NF)

Event Date:	09/30/2018
Event Type:	Regulatory - Meeting with FDA (Clinical Analysis)
Trial Name:	N/A
Market Group:	Neurology
Lead Company:	SpringWorks Therapeutics, LLC
Partner Companies:	N/A
Phase:	II
Change to Likelihood of Approval:	0%
Likelihood of Approval:	17% (Same As Avg.)
Average Approval:	17%

Analysis:

SpringWorks announced the company had a FDA pre-IND meeting in the third quarter of 2018. The FDA requested inclusion of both adult and pediatric patients in the upcoming Phase IIb trial, potentially enabling approval in both age cohorts. The study will be a single-arm, open-label study that may serve as a registrational study based on FDA precedent. SpringWorks anticipates the Phase IIb study to start in the first half of 2019.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (SpringWorks, Slide 16)

Nirogacestat for Solid Tumors

Event Date:	01/07/2019
Event Type:	Trial Data - Top-Line Results (Clinical Analysis)
Trial Name:	Phase I - Advanced Solid Tumors , Phase II - National Cancer Institute
Market Group:	Oncology
Lead Company:	SpringWorks Therapeutics, LLC
Partner Companies:	N/A
Phase:	II
Change to Likelihood of Approval:	0%
Likelihood of Approval:	10% (Same As Avg.)
Average Approval:	10%

Analysis:

SpringWorks presented data from the Phase I and Phase II study of Nirogacestat for the treatment of desmoid tumors at the 37th Annual J.P. Morgan Healthcare Conference.

Context

SpringWorks anticipates the Phase III study of nirogacestat in patients with desmoid tumors to begin in the first half of 2019. The company had an agreement with the FDA that progression-free survival (PFS) will serve as a primary endpoint of the study.

Design

Phase I

The Phase I study was conducted in 64 patients with advanced solid tumors to define the maximum tolerated dose; of these 64 patients, seven evaluable patients had a diagnosis of desmoid tumor.

Phase II

The Phase II study was an open-label, investigator-initiated study sponsored by National Cancer Institute (NCI) that enrolled 17 desmoid tumor patients with advanced or refractory tumors.

Results

Median PFS was not reached for both studies.

Phase I

The response rate by RECIST 1.0 was 5/7 PR (71.4%).

Phase II

The response rate by RECIST 1.0 was 5/17 PR (29.4%).

Most Common Adverse Events

Phase I

No discontinuations of the evaluable desmoid tumor patients. Two desmoid patients discontinued at

cycle 1 (rash, hypersensitivity) and were unable to be evaluated by tumor response. Grade 3 diarrhea was experienced by 6/64 (9.4%) and was generally manageable with anti-diarrheal therapy, dose interruption, or dose reduction. The only other grade 3 event occurring in >5% of patients with hypophosphatemia (15/64 pts, 23.4%) and was reversible with oral supplementation.

Phase II
One patient discontinuation due to grade 2 urticaria. Three other patients met the criteria for dose reductions and their symptoms resolved fully following dose reductions. The only grade 3 toxicity attributable to study drug was hypophosphatemia, which was experienced by 8/17 patients (47.1%) and can be easily reversed with oral phosphate replacement and without secondary symptoms of hypocalcemia or hypomagnesaemia. Only grade 1 and 2 diarrhea and skin disorders occurred.

Source:

Company Website (SpringWorks, Pipeline)
J.P. Morgan Healthcare Conference 01/07/2019 (SpringWorks, Slide 27)
Journal Article 01/31/2015 (*Clinical Cancer Research*; DOI: 10.1158/1078-0432.CCR-14-0607)
Journal Article 03/28/2017 (*Journal of Clinical Oncology*; DOI: 10.1200/JCO.2016.71.1994)
Journal Article 09/08/2017 (*Annals of Surgical Oncology*; DOI: 10.1245/s10434-017-6082-1)

PD-0325901 for Neurofibromatosis (NF)

Event Date:	01/07/2019
Event Type:	Trial Data - Top-Line Results (Clinical Analysis)
Trial Name:	Phase II - NF1-Associated Morbid Plexiform Neurofibromas
Market Group:	Neurology
Lead Company:	SpringWorks Therapeutics, LLC
Partner Companies:	N/A
Phase:	II
Change to Likelihood of Approval:	0%
Likelihood of Approval:	17% (Same As Avg.)
Average Approval:	17%

Analysis:

SpringWorks presented Phase II data for PD-0325901 in patients with NF1-associated PN at the 37th Annual J.P. Morgan Healthcare Conference.

Context
The company anticipates to commence a potential registrational trial in the first half of 2019 following the Phase II trial.

Design
19 patients with inoperable and symptomatic or growing PNs, ages 16-39 years (median age:24). 2mg/m^2 (up 4 mg BID) administered via intermittent (3 week on/1 week off) dosing schedule. 8/19

(42%) responders, prospectively defined as ≥20% tumor reduction by course 12 of treatment.

Endpoints
PR defined as ≥20% reduction from BL by volumetric MRI analysis.

Results
The PR rate was 8/19 PR (42.1%).

Most Common Adverse Events
No patient discontinuations, 5 dose reductions (1 due to grade 3 pain and the rest for grade 1-2 nausea, rash or fatigue); the most common DLT was acneiform rash. No grade 4 events, 5 grade 3 events (4 for pain, only 1 attributed to drug per investigator). Dose and schedule minimized historical class toxicities observed with other MEK inhibitors.

Source:

J.P. Morgan Healthcare Conference (SpringWorks, Slide 26)
Journal Article 06/18/2018 (*Neuro-Oncology*; DOI: 10.1093/neuonc/noy059.514)

PF-04457845 for Neurology - Other

Event Date:	01/07/2019
Event Type:	Progress Update (Clinical Analysis)
Trial Name:	N/A
Market Group:	Neurology
Lead Company:	SpringWorks Therapeutics, LLC
Partner Companies:	N/A
Phase:	Preclinical
Change to Likelihood of Approval:	N/A
Likelihood of Approval:	0% (Same As Avg.)
Average Approval:	N/A

Analysis:

SpringWorks lists a PF-04457845 combination therapy for the treatment of an undisclosed CNS indication in preclinical development in the company's pipeline. A Phase I study is anticipated to commence in the first quarter of 2020.

Source:

J.P. Morgan Healthcare Conference (SpringWorks, Slide 22)

Nirogacestat for Solid Tumors

Event Date:	03/31/2019
Event Type:	Regulatory - Meeting with FDA (Clinical Analysis)
Trial Name:	N/A
Market Group:	Oncology
Lead Company:	SpringWorks Therapeutics, LLC
Partner Companies:	N/A
Phase:	II
Change to Likelihood of Approval:	0%
Likelihood of Approval:	10% (Same As Avg.)
Average Approval:	10%

Analysis:

SpringWorks announced the company had an end of Phase II meeting with the FDA in the first quarter of 2018. The company had an agreement with the FDA for progression-free survival (PFS) to serve as the primary endpoint for a Phase III study. In the Phase I and Phase II trials, median PFS was not met due to lack of patients progressing on nirogacestat. The Phase III trial is expected to start in the first half of 2019.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (SpringWorks, Slide 10)

United Therapeutics Corporation (UTHR)

Orenitram for Pulmonary Arterial Hypertension (PAH) and Pulmonary Hypertension (PH)

Event Date:	12/21/2018
Event Type:	Regulatory - sNDA/sBLA Filing (Clinical Analysis)
Trial Name:	N/A
Market Group:	Cardiovascular
Lead Company:	United Therapeutics Corporation (UTHR)

Partner Companies:	Supernus Pharmaceuticals (SUPN) HealthCare Royalty Partners
Phase:	Approved
Change to Likelihood of Approval:	0%
Likelihood of Approval:	100% (Same As Avg.)
Average Approval:	100%

Analysis:

United Therapeutics announced that based on results from the FREEDOM-EV study of Orenitram for the treatment of pulmonary hypertension (PH) WHO Group 1 patients, the Company has filed for a label expansion. The filing occurred prior to the U.S. government shutdown, which began on December 22, 2018.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (UTHR)

Vertex Pharmaceuticals Incorporated (VRTX)

Alpha-1 Antitrypsin Program (Vertex) for Metabolic - General

Event Date:	12/31/2018
Event Type:	Progress Update (Clinical Analysis)
Trial Name:	N/A
Market Group:	Metabolic
Lead Company:	Vertex Pharmaceuticals Incorporated (VRTX)
Partner Companies:	N/A
Phase:	I
Change to Likelihood of Approval:	N/A
Likelihood of Approval:	16% (Same As Avg.)
Average Approval:	16%

Analysis:

Vertex announced the clinical development of the first small molecule corrector for Alpha-1 Antitrypsin Deficiency was initiated in December 2018.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (VRTX, Slide 16)

Alpha-1 Antitrypsin Program (Vertex) for Metabolic - General

Event Date:	01/07/2019
Event Type:	Trial Data - Preclinical Results (Clinical Analysis)
Trial Name:	Preclinical Studies
Market Group:	Metabolic
Lead Company:	Vertex Pharmaceuticals Incorporated (VRTX)
Partner Companies:	N/A
Phase:	I
Change to Likelihood of Approval:	0%
Likelihood of Approval:	16% (Same As Avg.)
Average Approval:	16%

Analysis:

Vertex announced preclinical findings for its small molecule corrector for Alpha-1 Antitrypsin Deficiency.

Context

Vertex announced the clinical development of the first small molecule corrector for Alpha-1 Antitrypsin Deficiency was initiated in December 2018.

Results

In a transgenic mouse model, functional AAT levels were increased 4.8 times after 4 days of treatment well into the carrier range.

Source:

J.P. Morgan Healthcare Conference 01/07/2019 (VRTX, Slide 16)

Biomedtracker/Meddevicetracker JPM New Catalysts - Day 1

Expected Date Range	Company	Symbol	Drug	Indication	Phase	Expected Catalyst	Catalyst Title	Analysis
01/07/2019-03/31/2019	Acceleron Pharma, Inc.	XLRN	Sotatercept	Pulmonary Arterial Hypertension	II	Trial Announcement - Initi	PHASE II - SPECTRA Trial to Star	Acceleron announced the Phase II SPECTRA study investigating sotatercept for the treatment of pulmonary arterial hypertension (PAH). The company plans for the study to initiate in the first quarter of 2019.
01/07/2019-03/31/2019	Ascendis Pharma A/S	ASND	TransCon PTH	Hypoparathyroidism	I	Trial Announcement - Initi	Phase II Adult Subjects - Study	Ascendis anticipates to initiate the Phase II trial in adult HP subjects in the first quarter of 2019.
01/07/2019-03/31/2019	Enanta Pharmaceuticals, I	ENTA	EDP-305	Non-Alcoholic Steatohepatitis (N	II	Trial Announcement - Pati	Phase II ARGON-1 - Enrollment	Enanta announced that enrollment in the 12-week Phase IIa NASH trial is expected to conclude in the first quarter of 2019.
01/07/2019-03/31/2019	Endo International plc	ENDP	Xiaflex	Cellulite	III	Partnership - Acquisition C	Acquisition Completion	Endo announces that the company plans for its acquisition of Somerset to complete in Q1 2019.
01/07/2019-04/30/2019	Jazz Pharmaceuticals plc	JAZZ	Solriamfetol	Excessive Sleepiness Associated	II	Trial Data - Top-Line Resul	Phase II - Top-Line Results	Jazz Pharmaceuticals expects top-line data in early 2019 for the Phase II study of Solriamfetol for EDS for PD.
Now-06/30/2019	Agios Pharmaceuticals, Inc	AGIO	AG-881	Brain Cancer (Malignant Glioma	I	Trial Data - Updated Resul	Phase I - Updated Results	Agios expects to announce perioperative data from the Phase I study in the first half of 2019.
Now-06/30/2019	Agios Pharmaceuticals, Inc	AGIO	AG-270	Cancer	I	Trial Announcement - Initi	Phase I - Dose Expansion Arms	Agios plans to initiate the dose expansion arms of the Phase I study of AG-270 including a single-agent arm in a variety of MTAP-deleted cancers and a combination arm in a solid tumor in the first half of 2019.
Now-06/30/2019	Agios Pharmaceuticals, Inc	AGIO	AG-636	Hematologic Cancer	Preclinical	Trial Announcement - Initi	Phase I to Start	Agios plans to initiate the Phase I study of AG-636 for the treatment of refractory lymphoma in the first half of 2019.
Now-06/30/2019	Agios Pharmaceuticals, Inc	AGIO	Tibsovo	Acute Myelogenous Leukemia (A	Approved	Trial Data - Updated Resul	Phase I/II w/Azacitidine - Upda	Agios plans to announced updated results from the Phase I combo trial of Tibsovo with azacitidine in newly diagnosed AML patients in the first half of 2019.
Now-06/30/2019	Agios Pharmaceuticals, Inc	AGIO	Tibsovo	Biliary Tract Cancer	III	Trial Data - Top-Line Resul	Phase III ClarIDHy - Top-Line Re	Agios expects to have results from the Phase III ClarIDHy trial in the first half of 2019.
01/07/2019-06/30/2019	Array BioPharma, Inc.	ARRY	Braftovi	Colorectal Cancer (CRC)	III	Trial Data - Updated Resul	Phase III BEACON - Updated Re	Array expects interim results from the Phase III BEACON study in H1 2019.
01/07/2019-06/30/2019	Array BioPharma, Inc.	ARRY	Mektovi	Colorectal Cancer (CRC)	III	Trial Data - Updated Resul	Phase III BEACON - Updated Re	Array expects interim results from the Phase III BEACON study in H1 2019.
04/01/2019-06/30/2019	Ascendis Pharma A/S	ASND	TransCon Growth Hormon	Short Stature / Growth Hormone	III	Trial Data - Top-Line Resul	Phase III fliGHt - Top-Line Resul	Ascendis expects top-line results from the Phase III fliGHt trial in the second quarter of 2019.
04/01/2019-06/30/2019	Ascendis Pharma A/S	ASND	TransCon Growth Hormon	Short Stature / Growth Hormone	III	Trial Announcement - Oth	Phase III enliGHten - Auto-Injec	Ascendis expects to introduce the auto-injector into the enliGHten trial in the second quarter of 2019.
01/07/2019-06/30/2019	Eli Lilly & Company	LLY	Erbitux	Colorectal Cancer (CRC)	Approved	Trial Data - Updated Resul	Phase III BEACON - Updated Re	Array expects interim results from the Phase III BEACON study in H1 2019.
01/07/2019-06/30/2019	Fresenius SE & Co. KGaA	FSNUY	Biosimilar Adalimumab (Fr	Psoriasis	III	Progress Update - Product	Product Launch (EU)	Fresenius announced that it expects to launch a biosimilar candidate to Humira (adalimumab) in the first half of 2019.
01/07/2019-06/30/2019	Fresenius SE & Co. KGaA	FSNUY	Biosimilar Adalimumab (Fr	Rheumatoid Arthritis (RA)	III	Progress Update - Product	Product Launch (EU)	Fresenius announced that it expects to launch a biosimilar candidate to Humira (adalimumab) in the first half of 2019.
04/01/2019-06/30/2019	Jazz Pharmaceuticals plc	JAZZ	Solriamfetol	Narcolepsy	NDA	Regulatory - Other	DEA Scheduling Decision	Jazz Pharmaceuticals expects a DEA scheduling decision for Solriamfetol in the second quarter of 2019.
04/01/2019-06/30/2019	Jazz Pharmaceuticals plc	JAZZ	Solriamfetol	Sleep Apnea	NDA	Regulatory - Other	DEA Scheduling Decision	Jazz Pharmaceuticals expects a DEA scheduling decision for Solriamfetol in the second quarter of 2019.
01/07/2019-06/30/2019	Jazz Pharmaceuticals plc	JAZZ	Xyrem	Narcolepsy	Approved	Progress Update - Product	Pediatric Launch	Jazz Pharmaceuticals expects to launch the pediatric indication for Xyrem in the first half of 2019.
03/01/2019-06/30/2019	Jazz Pharmaceuticals plc	JAZZ	JZP-258	Narcolepsy	III	Trial Data - Top-Line Resul	Phase III (Cataplexy) - Top-Line	Jazz Pharmaceuticals expects top-line data in spring 2019 for the Phase III study of JZP-258 for cataplexy for narcolepsy.
01/07/2019-06/30/2019	Mallinckrodt plc	MNK	H.P. Acthar Gel	Rheumatoid Arthritis (RA)	Approved	Trial Data - Updated Resul	Phase IV - Updated Results	Mallinckrodt announced that results for its Phase IV trial of H.P. Acthar Gel for the treatment of rheumatoid arthritis (RA) are anticipated in the first half of 2019.
01/07/2019-06/30/2019	Regeneron Pharmaceutica	REGN	Libtayo	Skin Cancer - Squamous Cell Car	Approved	Trial Announcement - Initi	Phase III Adjuvant Trial to Start	Regeneron plans to initiate a Phase III adjuvant trial of Libtayo in CSCC in the first half of 2019.
04/01/2019-07/31/2019	Neurocrine Biosciences, In	NBIX	NBI-74788	Congenital Adrenal Hyperplasia	II	Regulatory - Meeting with	Meeting with FDA	Pending a U.S. Food and Drug Administration (FDA) discussion in the second quarter Neurocrine expects a Phase III study in adults to initiate in the second half of 2019. We await an update on the details of the meeting in the time frame above.
04/01/2019-09/30/2019	Alnylam Pharmaceuticals,	ALNY	Onpattro	Hereditary Transthyretin (hATTR	Approved	Trial Announcement - Initi	Phase III - APOLLO B Trial to sta	At the 37th annual J.P. Morgan Healthcare Investor Conference Alnylam announced the Phase III - APOLLO B study investigating Onpattro for the treatment of patients with hereditary transthyretin (hATTR) Amyloidosis. The study is expected to initiate in mid-2019.
04/01/2019-09/30/2019	Enanta Pharmaceuticals, I	ENTA	EDP-938	Respiratory Syncytial Virus (RSV)	I	Trial Data - Top-Line Resul	Phase IIa - Top-Line Results	Enanta announced that the Phase IIa human challenge study of EDP-938 is advancing and top-line data are now expected mid-2019.
04/01/2019-09/30/2019	Genmab A/S	GEN:DC	Tisotumab Vedotin	Cervical Cancer	II	Trial Announcement - Pati	Phase II innovaTV 204 - Patient	Seattle Genetics announced they anticipate completing enrollment of the innovaTV 204 trial in mid-2019.

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Expected Date Range	Company	Symbol	Drug	Indication	Phase	Expected Catalyst	Catalyst Title	Analysis
07/01/2019-09/30/2019	Heron Therapeutics, Inc.	HRTX	HTX-011	Postsurgical Pain	NDA	Progress Update - Product	Product Launch	Heron expects HTX-011 to launch in the third quarter of 2019 if approved.
04/01/2019-09/30/2019	Spark Therapeutics, Inc.	ONCE	SPK-8011	Hemophilia A	III	Trial Data - Updated Resul	Phase I/II - Updated Results	Spark Therapeutics intends to report data for the Phase I/II study of SPK-8011 in hemophilia A in mid-year 2019.
07/01/2019-09/30/2019	SpringWorks Therapeutics, LLC		PD-0325901	Solid Tumors	I	Trial Announcement - Initi	Phase I/II Combo Study to Start	SpringWorks anticipates a Phase I/II with a PD-0325901 combination therapy to start in the third quarter of 2019.
10/01/2019-10/21/2019	United Therapeutics Corpo	UTHR	Orenitram	Pulmonary Arterial Hypertension	Approved	Regulatory - PDUFA for sN	PDUFA for sNDA - First Review	United Therapeutics announced that based on results from the FREEDOM-EV study of Orenitram for the treatment of pulmonary hypertension (PH) WHO Group 1 patients the Company has filed for a label expansion. The filing occurred prior to the U.S. government shutdown which began on December 22 2018. Based on a standard 10-month supplemental NDA filing under PDUFA V guidelines the
10/01/2019-10/31/2019	Agios Pharmaceuticals, Inc	AGIO	Tibsovo	Acute Myelogenous Leukemia (A	Approved	Regulatory - PDUFA for sN	PDUFA for sNDA - First Review	Agios announced that it has submitted a sNDA for the approval of Tibsovo for the treatment of untreated AML in December 2018. Based on a standard 10-month supplemental NDA filing under PDUFA V guidelines the PDUFA decision should occur in October 2018.
01/07/2019-12/31/2019	Acceleron Pharma, Inc.	XLRN	Luspatercept	Thalassemia	III	Trial Data - Published Resu	PHASE III - BELIEVE Published R	Acceleron announces that it plans to submit the results from the Phase III BELIEVE and MEDALIST studies for publication in 2019.
01/07/2019-12/31/2019	Acceleron Pharma, Inc.	XLRN	Luspatercept	Myelodysplastic Syndrome (MDS	III	Trial Data - Published Resu	PHASE III - MEDALIST Published	Acceleron announces that it plans to submit the results from the Phase III BELIEVE and MEDALIST studies for publication in 2019.
07/01/2019-12/31/2019	Acceleron Pharma, Inc.	XLRN	Luspatercept	Myelofibrosis (MF)	II	Trial Data - Top-Line Resul	Phase II Top-Line Results	Acceleron announces that it expects to release top-line results of its Phase II - MF-001 study investigating luspatercept for myelofibrosis in the second half of 2019.
10/01/2019-12/31/2019	Agios Pharmaceuticals, Inc	AGIO	Tibsovo	Biliary Tract Cancer	III	Regulatory - sNDA/sBLA Fi	sNDA Filing	Agios expects to submit a sNDA for Tibsovo for cholangiocarcinoma by the end of 2019.
10/01/2019-12/31/2019	Agios Pharmaceuticals, Inc	AGIO	AG-881	Brain Cancer (Malignant Glioma	I	Trial Announcement - Initi	Phase III to Start	Agios announced that the Phase III study of vorasidenib for low-grade glioma is expected to start by year-end 2019.
01/07/2019-12/31/2019	Agios Pharmaceuticals, Inc	AGIO	Mitapivat	Metabolic - General	III	Trial Announcement - Initi	Phase III - Patient Enrollment C	Agios' goal is to complete enrollment of its Phase III ACTIVATE trials in 2019.
07/01/2019-12/31/2019	Agios Pharmaceuticals, Inc	AGIO	Tibsovo	Biliary Tract Cancer	III	Trial Data - Final Results	Phase III ClarIDHy - Full Results	Agios expects to present full data from its Phase III ClarIDHy study of TIBSOVO in the second half of 2019.
07/01/2019-12/31/2019	Agios Pharmaceuticals, Inc	AGIO	AG-270	Cancer	I	Trial Data - Top-Line Resul	Phase I - Top-Line Results	Agios plans to have data from its Phase I dose-escalation trial of AG-270 in the second half of 2019.
07/01/2019-12/31/2019	Allergan plc	AGN	Novadur	Geographic Atrophy	II	Trial Announcement - Initi	Phase III - Trial to Start	Allergan reported that the Phase III study of brimonidine DDS in geographic atrophy is expected to start in the second half of 2019.
07/01/2019-12/31/2019	Allergan plc	AGN	CoolSculpting	Fat Removal	Approved	Regulatory - 510(k) Clearar	510(k) Approval Decision	Allergan reported that it is expecting the 510(k) clearance for CoolSculpting for muscle stimulation in the second half of 2019.
09/30/2019-12/31/2019	Ascendis Pharma A/S	ASND	TransCon PTH	Hypoparathyroidism	I	Trial Data - Top-Line Resul	Phase II - Top-Line Results	Ascendis expects top-line results for the Phase II trial in adult HP subjects during the fourth quarter of 2019.
01/07/2019-12/31/2019	Astellas Pharma, Inc.	4503:JP	Enfortumab Vedotin	Bladder Cancer	III	Regulatory - NDA/BLA Filin	BLA Filing	Seattle Genetics announced at the JP Morgan Healthcare conference that they anticipate a BLA filing for enfortumab vedotin in 2019.
01/07/2019-12/31/2019	AstraZeneca PLC	AZN	AZD4635	Solid Tumors	I	Trial Data - Top-Line Resul	Phase Ib w/Durvalumab - Top-L	Sosei announced it has been notified by its strategic alliance partner AstraZeneca that it has reached a clinical development milestone with its partnered next generation immuno-oncology candidate AZD4635. The candidate has been advancing through a Phase I clinical program as a single agent and in combination with AstraZeneca's anti-PD-L1 antibody durvalumab in patients with solid tumors. Headline data from the Phase I study is planned to be presented at a scientific congress in 2019. The company expects the study to be completed in 2020.
01/07/2019-12/31/2019	Bausch Health Companies	BHC	IDI-131	Psoriasis	Preclinical	Trial Announcement - Initi	Clinical Trial to Start	Bausch plans to initiate proof-of-concept development of IDI-131 in 2019.
Now-12/31/2019	Bausch Health Companies	BHC	IDP-123	Acne	III	Regulatory - NDA/BLA Filin	NDA Filing	Bausch plans to file for approval of IDP-123 for the treatment of Acne in 2019.
07/01/2019-12/31/2019	Biogen, Inc.	BIIB	BIIB092	Progressive Supranuclear Palsy	II	Trial Data - Top-Line Resul	Phase II Top-Line Data	Biogen announced they plan to release trial data for their Phase II PASSPORT study in the second half of 2019.
Now-12/31/2019	Biogen, Inc.	BIIB	IONIS-SOD1Rx	Amyotrophic Lateral Sclerosis (A	I/II	Trial Announcement - Initi	Pivotal Trial Initiation	Ionis announced the pivotal phase III of IONIS-SOD1Rx is expected to begin in 2019.

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Expected Date Range	Company	Symbol	Drug	Indication	Phase	Expected Catalyst	Catalyst Title	Analysis
Now-12/31/2019	Endo International plc	ENDP	Xiaflex	Cellulite	III	Regulatory - NDA/BLA Filin	BLA Filing	Endo announced that it plans to file a BLA for CCH (Xiaflex) for the treatment of cellulite in the second half of 2019 followed by a commercial launch in the second half of 2020.
07/01/2019-12/31/2019	Frequency Therapeutics, Inc.		FX-322	Hearing Loss - General	I/II	Trial Announcement - Initi	Phase II Study to Start	Frequency anticipates a Phase II study for FX-322 to initiate in the second half of 2019.
07/01/2019-12/31/2019	Heron Therapeutics, Inc.	HRTX	HTX-034	Postsurgical Pain	Preclinical	Trial Announcement - Initi	Phase II Study to Start	Heron expects the initiation of the Phase II study of HTX-034 in the second half of 2019.
01/07/2019-12/31/2019	Jazz Pharmaceuticals plc	JAZZ	Defitelio	Sinusoidal Obstruction Syndrom	Approved	Trial Data - Top-Line Resul	Phase III - Top-Line Results	Jazz Pharmaceuticals expects an interim analysis in 2019 for the Phase III study of Defitelio in high risk VOD patients to determine the final enrollment goal of 400 or up to 600 patients.
01/07/2019-12/31/2019	Jazz Pharmaceuticals plc	JAZZ	Defitelio	Drug Toxicity	Preclinical	Trial Announcement - Initi	Phase II Study to Start	Jazz Pharmaceuticals plans to initiate an exploratory Phase II study of Defitelio in 2019 for the prevention of CAR-T associated neurotoxicity in approximately 35 patients.
01/07/2019-12/31/2019	Jazz Pharmaceuticals plc	JAZZ	Defitelio	Graft vs. Host Disease (GVHD)	III	Trial Announcement - Pati	Phase II - Enrollment Complete	Jazz Pharmaceuticals expects to complete enrollment in the prevention of aGvHD Phase II study of Defitelio in 2019.
01/07/2019-12/31/2019	Jazz Pharmaceuticals plc	JAZZ	Vyxeos	Acute Myelogenous Leukemia (A	Approved	Trial Data - Updated Resul	Phase II - Updated Results	Jazz Pharmaceuticals expects to obtain data read outs in 2019 for Vyxeos in the COG R/R pediatric AML study and interim combination data from the MD Anderson collaboration.
10/01/2019-12/31/2019	Karyopharm Therapeutics	KPTI	Selinexor	Multiple Myeloma (MM)	NDA	Regulatory - Approval Dec	European Regulatory Approval	Karyopharm anticipates the European Medicines Agency (EMA) to approve selinexor in multiple myeloma at the end of 2019.
01/07/2019-12/31/2019	Kiniksa Pharmaceuticals C	KNSA	Mavrilimumab	Giant Cell Arteritis	II	Progress Update - Develop	Development Review - New Ind	Kiniksa plans to announce one additional indication to be evaluated for mavrilimumab in 2019.
07/01/2019-12/31/2019	Kiniksa Pharmaceuticals C	KNSA	KPL-716	Atopic Dermatitis (Eczema)	I	Trial Data - Updated Resul	Phase Ia/b - Updated Results (P	Kiniksa plans to release data from a repeated-single-dose Phase Ib study of KPL-716 for the treatment of atopic dermatitis in the second half of 2019.
01/07/2019-12/31/2019	Mallinckrodt plc	MNK	VTS-270	Niemann-Pick Disease	II/III	Trial Data - Updated Resul	Phase IIb/III - Updated Results	Mallinckrodt announced results from a study of VTS-270 for the treatment of Niemann-Pick Disease are anticipated in 2019.
07/01/2019-12/31/2019	Mallinckrodt plc	MNK	MNK-6105 IV/MNK-6106 (	Hepatic Encephalopathy (HE)	IIb	Trial Data - Top-Line Resul	Phase IIa (Oral) - Top-Line Resu	oral MNK-6106 for the treatment of hepatic encephalopathy (HE) are anticipated in the second half of
01/07/2019-12/31/2019	Mallinckrodt plc	MNK	MNK-6105 IV/MNK-6106 (	Hepatic Encephalopathy (HE)	IIb	Trial Announcement - Initi	Phase III MNK-6105 IV Trial to S	A Phase III study of MNK-6105 IV for hepatic encephalopathy (HE) is expected to start in 2019.
07/01/2019-12/31/2019	Mallinckrodt plc	MNK	Stanate	Hyperbilirubinemia	III	Trial Announcement - Initi	Phase III Study to Start	Mallinckrodt expects to initiate a follow on Phase III study of stannsoporfin for hyperbilirubinemia in 2019 in response to the CRL received in 2018. Mallinckrodt intends to work with the FDA on a SPA for this trial potentially in the second half of 2019.
09/01/2019-12/31/2019	Merck & Co., Inc.	MRK	Keytruda	Breast Cancer	III	Trial Data - Top-Line Resul	Phase III KEYNOTE-522 - Top-Li	Merck announced that interim data from the -522 study of Keytruda for the treatment of breast cancer are expected in late 2019.
01/07/2019-12/31/2019	Nabriva Therapeutics plc	NBRV	Lefamulin	Community Acquired Pneumoni	NDA	Trial Announcement - Initi	Pivotal Study to Start	Roivant Sciences expects the initiation of pivotal trials of lefamulin in community-acquired bacterial pneumonia in 2019.
Now-12/31/2019	Otsuka Holdings Co., Ltd.	4768:JP	Rexulti	Bipolar Disorder	III	Trial Data - Top-Line Resul	Phase III - Top-Line Results	Lundbeck expects pivotal data for Rexulti in bipolar mania in 2019.
Now-12/31/2019	Otsuka Holdings Co., Ltd.	4768:JP	Rexulti	Bipolar Disorder	III	Regulatory - sNDA/sBLA Fi	sNDA Filing	Lundbeck expects regulatory filing for Rexulti for bipolar mania in 2019.
Now-12/31/2019	Otsuka Holdings Co., Ltd.	4768:JP	Rexulti	Bipolar Disorder	III	Regulatory - Supplementat	Supplemental Filing (EU)	Lundbeck expects regulatory filing for Rexulti for bipolar mania in 2019.
Now-12/31/2019	Radius Health, Inc.	RDUS	Elacestrant	Breast Cancer	III	Trial Announcement - Initi	Combination Study - Trial to St	Radius expects a combination study of Elacestrant for the treatment of ER+ breast cancer in 2019.
Now-12/31/2019	Radius Health, Inc.	RDUS	Elacestrant	Breast Cancer	III	Partnership - New	Partnership - New	Radius anticipates the company will enter a global partnership for Elacestrant in 2019.
Now-12/31/2019	Radius Health, Inc.	RDUS	RAD140	Breast Cancer	I	Trial Data - Top-Line Resul	Phase Ia - Top-Line Results	Radius expects the next step for RAD140 is its Phase Ia results. We await an update on the readout of the trial in the time frame above.
01/07/2019-12/31/2019	Regeneron Pharmaceutica	REGN	Eylea	Wet Age-Related Macular Degen	Approved	Trial Announcement - Initi	High-Dose Eylea Clinical Trial to	Regeneron is advancing next-generation ophthalmology treatments such as a high-dose formulation of EYLEA which is expected to enter clinical trials in 2019.
01/07/2019-12/31/2019	Regeneron Pharmaceutica	REGN	Dupixent	Atopic Dermatitis (Eczema)	Approved	Trial Data - Top-Line Resul	Phase III - Pediatric Top-Line Re	Regeneron expects results from the Phase III study of Dupixent for atopic dermatitis in pediatrics in 2019.
07/01/2019-12/31/2019	Regeneron Pharmaceutica	REGN	REGN1979	Diffuse Large B-Cell Lymphoma (	Preclinical	Trial Announcement - Initi	Phase II to Start	Regeneron anticipates its Phase II study of REGN1979 in Diffuse Large B-Cell Lymphoma to begin in the second half of 2019.
01/07/2019-12/31/2019	Regeneron Pharmaceutica	REGN	REGN3500	Asthma	II	Trial Data - Top-Line Resul	Phase II - Top-Line Results	Regeneron plans to report results from the Phase II study of REGN3500 for the treatment of asthma in 2019.
01/07/2019-12/31/2019	Regeneron Pharmaceutica	REGN	Trevogrumab	Sarcopenia	II	Trial Data - Updated Resul	Phase I w/Garetosmab - Updat	Regeneron plans to report results from the multi-dose portion of the PHase I study of Trevogrumab and Garetosmab in 2019.
01/07/2019-12/31/2019	Regeneron Pharmaceutica	REGN	Garetosmab	Fibrodysplasia Ossificans Progre	II	Trial Data - Updated Resul	Phase I w/Trevogrumab - Upda	Regeneron plans to report results from the multi-dose portion of the PHase I study of Trevogrumab and Garetosmab in 2019.
01/07/2019-12/31/2019	Regeneron Pharmaceutica	REGN	Libtayo	Hematologic Cancer	I	Trial Announcement - Initi	Phase I - 1st Line Classical Hodg	Regeneron plans to initiate the Phase I study of Libtayo for the first-line treatment of classical Hodgkin lymphoma in 2019.
01/07/2019-12/31/2019	Roivant Sciences, Inc.		Tapinarof	Psoriasis	IIb	Trial Announcement - Initi	Phase III Studies to Start	Roivant Sciences expects the initiation of two Phase III trials of tapinarof for psoriasis in 2019.
01/07/2019-12/31/2019	Roivant Sciences, Inc.		RVT-1401	Myasthenia Gravis (MG)	Development Outsi	Trial Data - Top-Line Resul	Phase IIa - Top-Line Results	Roivant Sciences expects top-line data from a Phase IIa study of RVT-1401 in myasthenia gravis in 2019.
07/01/2019-12/31/2019	Rubius Therapeutics	RUBY	RTX-134	Phenylketonuria (PKU)	Preclinical	Trial Data - Top-Line Resul	Phase Ib/IIa Top-Line Results	Rubius expects initial data from Part 1 of the Phase Ib/IIa trial of RTX-134 in adult patients with PKU.

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Expected Date Range	Company	Symbol	Drug	Indication	Phase	Expected Catalyst	Catalyst Title	Analysis
09/01/2019-12/31/2019	United Therapeutics Corp	UTHR	Unituxin	Small Cell Lung Cancer (SCLC)	II/III	Trial Data - Top-Line Result	Phase II/III DISTINCT - Top-Line	United Therapeutics announced that top-line results from the Phase II/III DISTINCT study of Unituxin for the treatment of small cell lung cancer are anticipated later in 2019.
09/01/2019-03/31/2020	Agios Pharmaceuticals, Inc	AGIO	Tibsovo	Acute Myelogenous Leukemia (A	Approved	Regulatory - CHMP (Europ	CHMP Opinion	Agios submitted an MAA for approval in Europe of Tibsovo for AML in December 2018. Based on an internal analysis of the centralized European approval procedure we estimate the European marketing authorization for this drug for this indication will be granted in approximately 11-17 months. As the approval decision is normally issued 67 days from adoption of a positive Committee for Medicinal Products for Human Use (CHMP) opinion we then estimate the CHMP opinion to occur between September 2019 and March 2020.
04/01/2019-03/31/2020	Daiichi Sankyo Co., Ltd.	DSKYF	DS-8201	Solid Tumors	I	Trial Announcement - Initi	Phase Ib w/TKI Inhibitor - Trial	Daiichi Sankyo expects to initiate a Phase Ib trial of DS-8201 in combination with a TKI Inhibitor for the treatment of solid tumors in the company's fiscal year 2019.
09/01/2019-03/31/2020	Daiichi Sankyo Co., Ltd.	DSKYF	DS-8201	Gastric Cancer	Development Outsi	Trial Announcement - Initi	Phase III 2nd Line - Trial to Star	Daiichi Sankyo expects to initiate a Phase III 2nd line gastric cancer trial with DS-8201 vs. SOC in the second half of the company's fiscal year 2019.
01/07/2019-03/31/2020	Santen Pharmaceutical Co	4536:JP	Verkazia	Allergic Conjunctivitis (Ophthalm	Approved in Europe	Progress Update - Product	Product Launch - Canada	Santen expects to launch Verkazia in Canada in the company's fiscal year 2018 or 2019.
01/01/2020-03/31/2020	SpringWorks Therapeutics, LLC		PF-04457845	Neurology - Other	Preclinical	Trial Announcement - Initi	Phase I Combo Study to Start	SpringWorks anticipates to initiate a Phase I study for PF-04457845 combination therapy in the first quarter of 2020.
01/01/2020-04/30/2020	Incyte Corporation	INCY	INCB18424 (Topical)	Atopic Dermatitis (Eczema)	III	Trial Data - Top-Line Result	Phase III TRuE-AD2 - Top-Line R	Incyte announced data from the Phase III TRue AD studies of topical ruxolitinib are expected in early 2020.
01/01/2020-04/30/2020	Incyte Corporation	INCY	INCB18424 (Topical)	Atopic Dermatitis (Eczema)	III	Trial Data - Top-Line Result	Phase III TRuE-AD1 - Top-Line R	Incyte announced data from the Phase III TRue AD studies of topical ruxolitinib are expected in early 2020.
01/01/2020-04/30/2020	Rubius Therapeutics	RUBY	RTX-240	Solid Tumors	Preclinical	Regulatory - IND Filing	IND Filing	Rubius plans to file an Investigational New Drug application (IND) for RTX-240 for the treatment of solid tumors in early 2020.
11/01/2019-05/31/2020	Agios Pharmaceuticals, Inc	AGIO	Tibsovo	Acute Myelogenous Leukemia (A	Approved	Regulatory - Approval Dec	European Approval Decision	Agios submitted an MAA for approval in Europe of Tibsovo for AML in December 2018. Based on an internal analysis of the centralized European approval procedure we estimate the European marketing authorization for this drug for this
01/01/2020-06/30/2020	Frequency Therapeutics, Inc.		FX-322	Hearing Loss - General	I/II	Trial Data - Updated Result	Phase I/II - Updated Results	Frequency anticipates data readouts for the Phase IIa study of FX-322.
01/01/2020-06/30/2020	Frequency Therapeutics, Inc.		FX-322	Hearing Loss - General	I/II	Trial Announcement - Tria	Phase I/II - Trial Completion	Frequency anticipates to complete the Phase IIa trial of FX-322.
01/01/2020-06/30/2020	SpringWorks Therapeutics, LLC		PD-0325901	Solid Tumors	I	Trial Data - Top-Line Result	Phase Ib w/ Lifirafenib - Top-Lir	SpringWorks anticipates top-line results for the Phase Ib study of PD-0325901 + Lifirafenib for the treatment of solid tumors during the first half of 2020.
01/01/2020-12/31/2020	Acceleron Pharma, Inc.	XLRN	Luspatercept	Thalassemia	III	Trial Data - Top-Line Result	Phase II - BEYOND Top-Line Res	Acceleron announces that it expects to release top-line results of the Phase II - BEYOND trial investigating luspatercept for thelassemia in 2020.
01/01/2020-12/31/2020	Bausch Health Companies	BHC	IDP-124	Atopic Dermatitis (Eczema)	III	Regulatory - NDA/BLA Filin	NDA Filing	Bausch plans to submit approval of IDP-124 for the treatment of atopic dermatitis in 2020.
01/01/2020-12/31/2020	Bausch Health Companies	BHC	IDP-120	Acne	II	Regulatory - NDA/BLA Filin	NDA Filing	Bausch plans to file for approval of IDP-120 for the treatment of acne in 2020.
01/01/2020-12/31/2020	Endo International plc	ENDP	Xiaflex	Cellulite	III	Progress Update - Product	Product Launch (U.S.)	Endo announced that it plans to file a BLA for CCH (Xiaflex) for the treatment of cellulite in the second half of 2019 followed by a commercial launch in the second half of 2020.
01/01/2020-12/31/2020	Incyte Corporation	INCY	INCB54828	Bladder Cancer	II	Regulatory - sNDA/sBLA Fil	sNDA Filing	Incyte expects to submit a sNDA for INCB54828 as a treatment for patients with bladder cancer in 2020.
07/01/2020-12/31/2020	Mallinckrodt plc	MNK	H.P. Acthar Gel	Sarcoidosis	Approved	Trial Data - Top-Line Result	Phase IV - Top-Line Results	Mallinckrodt announced that results for its Phase IV trial of H.P. Acthar Gel for the treatment of sarcoidosis are anticipated in the second half of 2020.
07/01/2020-12/31/2020	Mallinckrodt plc	MNK	H.P. Acthar Gel	Uveitis (Ophthalmology)	Approved	Trial Data - Top-Line Result	Phase IV - Top-Line Results	Mallinckrodt announced that results for its Phase IV trial of H.P. Acthar Gel for the treatment of uveitis are anticipated in the second half of 2020.
07/01/2020-12/31/2020	Mallinckrodt plc	MNK	H.P. Acthar Gel	Focal Segmental Glomeruloscler	III	Trial Data - Top-Line Result	Phase IV - Top-Line Results	Mallinckrodt announced that results for its Phase IV trial of H.P. Acthar Gel for the treatment of FSGS are anticipated in the second half of 2020.
01/01/2020-12/31/2020	Radius Health, Inc.	RDUS	Tymlos	Osteoporosis / Osteopenia	Approved	Trial Data - Top-Line Result	Phase III ATOM - Top-Line Resu	Radius expects results from the Phase III trial of abaloparatide-SC in men with osteoporosis in 2020.
01/01/2020-12/31/2020	United Therapeutics Corp	UTHR	Tyvaso	Pulmonary Arterial Hypertension	Approved	Trial Data - Top-Line Result	Phase II/III INCREASE - Top-Line	United Therapeutics announced that they plan to release top-line results of the Phase II/III INCREASE study of Tyvaso in 2020.
01/01/2020-12/31/2020	United Therapeutics Corp	UTHR	Orenitram	Pulmonary Arterial Hypertension	Approved	Trial Announcement - Pati	Phase III SOUTHPAW - Patient E	United Therapeutics announced that enrollment in the Phase III SOUTHPAW study is anticipated to complete in 2020.
04/01/2020-03/31/2021	Daiichi Sankyo Co., Ltd.	DSKYF	DS-8201	Breast Cancer	III	Regulatory - NDA/BLA Filin	BLA Filing	Daiichi Sankyo expects to file a BLA for DS-8201a in the company's fiscal year 2020.
01/01/2021-12/31/2021	AstraZeneca PLC	AZN	AZD4635	Non-Small Cell Lung Cancer (NSC	I/II	Trial Announcement - Tria	Phase Ib/II Completion	Sosei announced that the company plans for the Phase Ib/II trial investigating AZD4635 in combination for oleclumab for the treatment of non-small cell lung cancer to complete in 2021.

Biomedtracker/Meddevicetracker JPM New Catalysts - Day 1

Expected Date Range	Company	Symbol	Drug	Indication	Phase	Expected Catalyst	Catalyst Title	Analysis
01/01/2021-12/31/2021	Bausch Health Companies	BHC	IDP-133	Atopic Dermatitis (Eczema)	Preclinical	Regulatory - NDA/BLA Filin	NDA Filing	Bausch announced the development of IDP-133 a halobetasol propionate lotion for the treatment of atopic dermatitis in its pipeline. Bausch plans to submit for approval in 2021.
01/01/2021-12/31/2021	Bausch Health Companies	BHC	IDP-126	Acne	II	Regulatory - NDA/BLA Filin	NDA Filing	Bausch plans to file for approval of IDP-126 for the treatment of acne in 2021.
01/01/2021-12/31/2021	Radius Health, Inc.	RDUS	Elacestrant	Breast Cancer	III	Trial Data - Top-Line Result	Phase III EMERALD - Top-Line R	Radius expects data readout from the Phase III EMERALD study of Elacestrant in 2021.
01/01/2021-12/31/2021	United Therapeutics Corp	UTHR	Tyvaso	Pulmonary Arterial Hypertension	Approved	Trial Data - Top-Line Result	Phase III PERFECT - Top-Line Re	United Therapeutics announced that they plan to release top-line results of the Phase III PERFECT study of Tyvaso in 2021.
01/01/2022-06/30/2022	Mallinckrodt plc	MNK	MNK-6105 IV/MNK-6106 (	Hepatic Encephalopathy (HE)	IIb	Trial Data - Top-Line Result	Phase III (IV) - Top-Line Results	Mallinckrodt announced results from a Phase III study of IV MNK-6105 for the treatment of hepatic encephalopathy (HE) are anticipated in the first half of 2022.
01/01/2021-12/31/2022	Alnylam Pharmaceuticals,	ALNY	Vutrisiran	Hereditary Transthyretin (hATTR	III	Trial Data - Top-Line Result	Phase III - HELIOS-A Top-Line Re	Alnylam announced that it has initiated the Phase III - HELIOS-A study investigating vutrisiran for patients with hereditary transthyretin (hATTR) amyloidosis with polyneuropathy. Target enrollment is 160 participants. The company expects a data readout in 2021-2022.

Biomedtracker/Meddevicetracker JPM Updated Catalysts - Day 1

Expected Date Range	Company	Symbol	Drug	Indication	Phase	Expected Catalyst	Catalyst Title	Analysis	Updated Analysis
Now-01/01/2019	Seattle Genetics, Inc.	SGEN	Tucatinib	Breast Cancer	II	Trial Data - Top-Line Results	Phase II HER2CLIMB - Topline Results	Cascadian Therapeutics announced that the Company anticipates providing updated data for the ongoing Phase II study known as HER2CLIMB by the end of 2016	announced that two abstracts on the Company's lead product candidate tucatinib (also known as ONT-380) will be presented at the 2016 San Antonio Breast Cancer Symposium (SABCS). The abstract entitled A Phase 2 Randomized Double-Blinded Controlled Study of ONT-380 vs. Placebo in Combination with Capecitabine (C) and Trastuzumab (T) in Patients with Pretreated HER2+ Unresectable Locally Advanced or Metastatic Breast Carcinoma (MBC) (HER2CLIMB) will be presented on December 7 2016.Date Range Delayed (12/07/2016) - The abstract related to the HER2CLIMB trial that was presented at the 2016 San Antonio Breast Cancer Symposium did not contain top-line study results. We await an update.Date Range Delayed (01/05/2017) - Cascadian announced it will present new data analyses from tucatinib at scientific meetings in 2017.Date Range Delayed (12/05/2017) - Cascadian announced that enrollment of the Phase II HER2CLIMB study of Tucatinib is on track and enrolling in North America Western Europe and Australia. We await the announcement of top-line results through 2018.Date Range Delayed (07/11/2018) - Seattle Genetics previously announced that the HER2CLIMB trial a global randomized pivotal trial for patients with HER2-positive (HER2+) metastatic breast cancer including patients with or without brain metastases is expected to be fully enrolled in 2019. As such we expect data to be reported in 2019.New Information (01/07/2019) - Per Seattle Genetic's JP Morgan
Now-01/18/2019	Celgene Corporation	CELG	Revlimid	Diffuse Large B-Cell Lymphoma (DLBCL) - NHL	III	Trial Data - Top-Line Results	Phase III ROBUST - Top-Line Results	Celgene expects to have data from its Phase III ROBUST study of Revlimid in 2018.	Date Range Delayed (08/12/2016) - Celgene expects to have data from its Phase III ROBUST study of Revlimid in 2019. Date Range Expedited (01/09/2017) - Celgene expects to have data from its Phase III ROBUST study of Revlimid in 2018.Date Range Delayed (02/01/2017) - Celgene expects to have data from its Phase III ROBUST study of Revlimid in 2019.Date Range Expedited (08/01/2017) - Celgene announced that data from the Phase III ROBUST trial evaluating REVLIMID in combination with rituximab plus chemotherapy (R-CHOP) in patients with ABC type diffuse large B-cell lymphoma (DLBCL) are expected in 2018.Date Range Refined (01/08/2018) - Celgene plans to have data from its Phase III ROBUST study of Revlimid for DLBCL in the second half of 2018.Date Range Delayed (12/31/2018) - We await an update.Date Range Delayed (01/07/2019) - Data are expected in 2019 from the Phase III ROBUST trial with REVLIMID in
Now-01/18/2019	Celgene Corporation	CELG	Abraxane	Pancreatic Cancer	Approved	Trial Data - Top-Line Results	Phase III APACT - Top-Line Results	Celgene expects to have data from its Phase III APACT study of Abraxane in 2017.	New Information (05/02/2016) - Celgene announced that the Phase III apact trial evaluating ABRAXANE in combination with gemcitabine as adjuvant therapy in patients with surgically resected pancreatic cancer completed enrollment. Data from this trial are expected in 2017. Date Range Refined (01/30/2017) - Celgene announced that data from the Phase III APACT trial evaluating ABRAXANE in combination with gemcitabine as adjuvant therapy in patients with surgically resected pancreatic cancer is expected by year-end 2017.Date Range Expedited (08/01/2017) - Celgene expects data from the Phase III APACT (PANC-003) trial with ABRAXANE as adjuvant therapy in patients with surgically resected pancreatic cancer in the second half of 2017.Date Range Delayed (10/31/2017) - Celgene expects data from the Phase III APACT trial with ABRAXANE as adjuvant therapy in patients with surgically resected pancreatic cancer by year-end 2018.Date Range Delayed (12/31/2018) - We await an update.Date Range Delayed (01/07/2019) - Data from the Phase III apact trial with ABRAXANE as adjuvant therapy in patients with surgically resected pancreatic cancer (event-driven) is expected in 2019.
Now-01/18/2019	Celgene Corporation	CELG	Lisocabtagene Maraleucel	Chronic Lymphocytic Leukemia (CLL)/Small Cell Lymphocytic Lymphoma (SLL) - NHL	I/II	Trial Announcement - Initiation	Phase II w/Ibrutinib - Trial to Start	Juno announced that it expects to start a Phase II trial of JCAR017 with ibrutinib in CLL in the second half of 2018.	Date Range Delayed (12/31/2018) - We await an update.Date Range Delayed (01/07/2019) - A pivotal Phase II trial in relapsed/refractory chronic lymphocytic leukemia (CLL) is to be initiated in H1:2019 for Liso-cel.
Now-01/18/2019	Celgene Corporation	CELG	Lisocabtagene Maraleucel	Chronic Lymphocytic Leukemia (CLL)/Small Cell Lymphocytic Lymphoma (SLL) - NHL	I/II	Trial Announcement - Initiation	Pivotal Trial to Start	Juno announced the potential initiation of a pivotal trial with Liso-cel in r/r CLL in 2018.	Date Range Delayed (12/31/2018) - We await an update.Catalyst Removed (01/07/2019) - We are removing this duplicate catalyst.
Now-01/18/2019	Celgene Corporation	CELG	Otezla	Behçet Syndrome	NDA	Regulatory - Supplemental Filing (Japan)	Supplemental Filing (Japan)	Celgene plans to submit supplemental New Drug Applications for apremilast 30 mg BID for the treatment of active Behcet's Disease with oral ulcers in the U.S. and Japan in the second half of 2018.	Date Range Refined (11/27/2018) - A Japanese filing for Otezla in Behcet's disease is planned in Q4 2018.Date Range Delayed (12/31/2018) - We await an update.Catalyst Occurred (01/07/2019) - Approval is expected by the U.S. FDA for the OTEZLA sNDA in Beh?et's disease with a Prescription Drug User Fee Act (PDUFA) action date of July 21 2019. Approval by the PMDA in Japan is expected in the second half of 2019.
01/07/2019-01/31/2019	Agios Pharmaceuticals, Inc.	AGIO	Tibsovo	Biliary Tract Cancer	III	Trial Announcement - Patient Enrollment Completed	Phase III - ClarIDHy - Patient Enrollment Completed	Agios expects to complete enrolment of the Phase III ClarIDHy study of ivosidenib for cholangiocarcinoma in 2019 to support global registration.	Date Range Refined (06/08/2018) - Agio has accelerated the completion of enrollment of ClarIDHy a global registration-enabling randomized Phase III study for ivosidenib in IDH1m positive advanced cholangiocarcinoma to the first half of 2019.Date Range Delayed (01/07/2019) - Agios expects to complete enrollment of the study in the next couple of weeks. We await an update through the end of January 2019.
Now-01/31/2019	Karyopharm Therapeutics	KPTI	Selinexor	Multiple Myeloma (MM)	NDA	Regulatory - MAA Submission (Europe)	MAA Filing	Karyopharm plans to file an MAA/NDA for Selinexor in MM in the second half of 2017 based on results of the STORM study.	Date Range Delayed (10/12/2016) - Karyopharm plans to seek conditional approval assuming positive data from the STORM trial expansion cohort with a potential MAA submission in Mid-2018.Date Range Delayed (04/30/2018) - Karyopharm plans to submit a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) in early 2019 with a request for conditional approval for oral selinexor as a new treatment for patients with penta-refractory multiple myeloma. Date Range Refined (08/29/2018) - Karyopharm plans to submit a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) in Q1 2019 with a request for conditional approval.Date Range Refined (01/07/2019) - Karyopharm now says it plans to submit a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) in January 2019.

Biomedtracker/Meddevicetracker JPM Updated Catalysts - Day 1

Expected Date Range	Company	Symbol	Drug	Indication	Phase	Expected Catalyst	Catalyst Title	Analysis	Updated Analysis
01/07/2019-02/28/2019	Agios Pharmaceuticals, Inc.	AGIO	Tibsovo	Acute Myelogenous Leukemia (AML)	Approved	Trial Announcement - Initiation	Phase III - HOVON 15 AML/AMLSG 29-18 Trial Initiation	Agios announced that with support from Celgene an intergroup sponsored global registration-enabling Phase III trial combining ivosidenib or enasidenib (Idhifa) and standard induction (7+3) and consolidation chemotherapy in frontline AML patients with an IDH1 or IDH2 mutation is planned to initiate in the fourth quarter of 2018.	Date Range Delayed (12/31/2018) - We await an update.Date Range Delayed (01/07/2019) - Agios expects to initiate the trial in intensive chemotherapy patients with Tibsovo shortly. We await an update through February 2019.
03/16/2019-03/18/2019	Medtronic plc	MDT	TYRX Absorbable Antibacterial Envelope	Cardiac Valve Surgery	Approved	Trial Data - Top-Line Results	Phase IV WRAP-IT - Top-line Results	Medtronic announced it plans to release top-line results for the WRAP-IT clinical trial in fiscal year 2018.	New Information (01/09/2018) - Medtronic announced that it expects top-line results from the WRAP IT clinical trial during its 2018 or 2019 fiscal year.Date Range Refined (01/07/2019) - Medtronic announced that results from the WRAP-IT Study will be presented at the 2019 American College of Cardiology Scientific Sessions held from March 16-18 2019.
Now-03/31/2019	Astellas Pharma, Inc.	4503:JP	Enfortumab Vedotin	Bladder Cancer	III	Trial Data - Top-Line Results	Phase II EV-201 - Top-Line Results	Seattle Genetics and Astellas Pharma expect to report top-line efficacy and safety results from this first cohort of the EV-201 trial which is intended to support potential registration under the U.S. Food and Drug Administration's (FDA) accelerated approval pathway in the first half of 2019.	Date Range Refined (01/07/2019) - Seattle Genetics announced they plan to release the top-line data from this trial during Q1 of 2019.
01/07/2019-03/31/2019	BioMarin Pharmaceutical Inc.	BMRN	Palynziq	Phenylketonuria (PKU)	Approved	Regulatory - CHMP (European Panel) Results	CHMP Opinion	BioMarin announced that the European Medicines Agency (EMA) has accepted BioMarin's submission of a Marketing Authorization Application (MAA) for pegvaliase a PEGylated recombinant phenylalanine ammonia lyase enzyme product for the treatment of adults with phenylketonuria (PKU) who have inadequate blood phenylalanine control (blood phenylalanine levels greater than 600 micromol/l) despite prior management with available treatment options including sapropterin. Based on an internal analysis of the centralized European approval procedure we estimate the European marketing authorization for this drug for this indication will be granted in approximately 11-17 months. As the approval decision is normally issued 67 days from adoption of a positive Committee for Medicinal Products for Human Use (CHMP) opinion we then estimate the CHMP opinion to occur between December 2018 and June 2019.	Date Range Refined (01/07/2019) - BioMarin noted during the presentation that the company is anticipating an opinion from the Committee for Medicinal Products for Human Use (CHMP) the scientific committee of the European Medicines Agency (EMA) on Palynziq (pegvaliase) Injection for the treatment of patients 16 and older with Phenylketonuria (PKU) in the first quarter of 2019. If the CHMP provides a positive opinion in the first quarter of 2019 then in the second quarter of 2019 it is possible that the European Commission (EC) could provide marketing authorization in the European Union.
Now-03/31/2019	Celgene Corporation	CELG	Revlimid	Indolent Non-Hodgkin's Lymphoma (Including Follicular Lymphoma) - NHL	III	Regulatory - sNDA/sBLA Filing	sNDA Filing	Celgene plans to prepare global regulatory submissions for Revlimid for the treatment of relapsed/refractory follicular and marginal zone lymphoma in the first quarter of 2019.	Catalyst Occurred (01/07/2019) - Celgene announced that approval is expected by the U.S. Food and Drug Administration (FDA) on the supplemental New Drug Application (sNDA) for REVLIMID in combination with rituximab in relapsed/refractory indolent lymphoma (AUGMENT). The company confirmed that the REVLIMID sNDA was submitted. We suspect that the submission occurred in December 2018.
Now-03/31/2019	Mesoblast Limited	MESO	MPC-150-IM	Congestive Heart Failure (CHF) and Cardiomyopathies	III	Trial Announcement - Patient Enrollment Completed	Phase III - Patient Enrollment Completed	A Phase III trial of MPC-150-IM at 150 million cell dose in up to 600 patients with advanced heart failure is currently being evaluated. This trial is expected to complete enrollment at the end of 2018.	Date Range Expedited (03/30/2018) - Mesoblast expects enrollment to complete in the Phase III trial of NeoFuse in the second half of 2018.Date Range Delayed (12/28/2018) - Mesoblast expects to complete patient recruitment in the Phase III trial evaluating Revascor in patients with moderate-to-severe advanced chronic heart failure between the fourth quarter of 2018 and the first quarter of 2019.Catalyst Occurred (01/07/2019) - Mesoblast announced that it has completed patient recruitment in the events-driven Phase III trial of its product candidate Revascor (MPC-150-IM) for advanced chronic heart failure.
Now-03/31/2019	MyoKardia, Inc.	MYOK	Mavacamten	Congestive Heart Failure (CHF) and Cardiomyopathies	III	Trial Data - Updated Results	Phase II PIONEER-OLE - Updated Results	MyoKardia plans to present additional data from PIONEER-OLE in the first quarter of 2019 including gradient and ejection fraction data from longer term exposures NT-proBNP and NYHA functional classification changes.	New Information (01/07/2019) - Myokardia announces that the 6 month data from the Phase II - PIONEER-OLE trial were accepted for presentation at the American College of Cardiology meeting in March 2019.
Now-03/31/2019	Pfizer Inc.	PFE	PF-06838435	Hemophilia B	I/II	Trial Announcement - Initiation	Phase III - Trial to Start	Spark Therapeutics announced the company has completed enrollment in the Phase I/II clinical trial of SPK-9001 in hemophilia B and expects to complete the transition of the program to Pfizer this summer. Additionally Spark Therapeutics expects to deliver a batch of drug substance to Pfizer enabling Pfizer to begin a Phase III clinical trial. As such we await the start of a Phase III trial in the time frame above.	New Information (07/16/2018) - Pfizer and Spark Therapeutics announced that Pfizer initiated a Phase III lead-in study to evaluate the efficacy and safety of current factor IX prophylaxis replacement therapy in the usual care setting. The factor IX prophylaxis efficacy data obtained in the lead-in study will serve as the within-subject control group for those patients that enroll into the next part of the Phase III study which will evaluate the investigational gene therapy fidanacogene elaparvovec for the treatment of hemophilia B. As such we continue to await the start of a Phase III trial with fidanacogene elaparvovec in the time time frame above.Date Range Delayed (01/07/2019) - Spark Therapeutics announced that the Phase III clinical trial of SPK-9001 in hemophilia B is expected to start in 2019.
01/07/2019-03/31/2019	Regeneron Pharmaceuticals, Inc.	REGN	Dupixent	Nasal Polyposis	III	Regulatory - sNDA/sBLA Filing	sBLA Filing	Regeneron announced that an sBLA for Dupixent is planned for dupilumab in adults with nasal polyposis in the first half of 2019.	Date Range Refined (12/13/2017) - Sanofi announced that an sBLA for Dupixent is planned for dupilumab in adults with nasal polyposis in the first half of 2019. Date Range Refined (01/07/2019) - Regeneron plans to file a sBLA for Dupixent for the treatment of chronic rhinosinusitis with nasal polyps in the first quarter of 2019.
4/29/2019	Regeneron Pharmaceuticals, Inc.	REGN	Praluent	Dyslipidemia / Hypercholesterolemia	Approved	Regulatory - PDUFA for sNDA/sBLA	PDUFA for sBLA - First-Line Hyperlipidemia	Regeneron announced that an sBLA of Praluent for first-line treatment of hyperlipidemia has also been recently submitted in the second quarter of 2018. Based on a standard 10-month supplemental BLA filing under PDUFA V guidelines the PDUFA decision should occur between February and April 2019.	Exact Date (01/07/2019) - Regeneron expects a FDA decision on the sBLA for Praluent for first-line treatment of Hyperlipidemia on April 29 2019.
Now-04/30/2019	Celgene Corporation	CELG	Fedratinib	Myelofibrosis (MF)	III	Regulatory - NDA/BLA Filing	NDA Filing	Celgene plans to submit a New Drug Application to the FDA for fedratinib in myelofibrosis in 2018.	Date Range Refined (01/08/2018) - Based on the reported benefit risk profile of fedratinib from the JAKARTA-1 and JAKARTA-2 clinical trials regulatory applications in myelofibrosis are planned beginning in the middle of 2018.Date Range Delayed (09/20/2018) - Celgene announced that the U.S. NDA submission of fedratinib for myelofibrosis is on track for 2018.Date Range Delayed (12/31/2018) - We await an update through early 2019.Catalyst Occurred (01/07/2019) - Celgene announced that the U.S. FDA approval of fedratinib is expected by year-end 2019. The company confirmed that the fedratinib NDA was submitted.
01/07/2019-04/30/2019	Incyte Corporation	INCY	INCB18424 (Topical)	Vitiligo	II	Trial Data - Top-Line Results	Phase II - Top-Line Results	Incyte announced that Phase II data for topical ruxolitinib in vitiligo are expected in 2019.	Date Range Refined (01/07/2019) - Incyte announced that Phase II data for topical ruxolitinib in vitiligo are expected in early 2019.

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Expected Date Range	Company	Symbol	Drug	Indication	Phase	Expected Catalyst	Catalyst Title	Analysis	Updated Analysis
05/01/2019-05/31/2019	Allergan plc	AGN	Vraylar	Bipolar Disorder	Approved	Regulatory - PDUFA for sNDA/sBLA	PDUFA for sNDA - First Review	Allergan announced that the U.S. Food and Drug Administration (FDA) has accepted for review the company's supplemental New Drug Application (sNDA) for VRAYLAR (cariprazine) seeking to expand the indication to include the treatment of depressive episodes associated with bipolar I disorder (bipolar depression) in adults in the current product label. In accordance with federal regulation the FDA has 60 days from the date of receipt of the application to determine acceptability of the filing. Allowing for the standard 10 month supplemental review period the estimated PDUFA date would fall between May 2019 and June 2019.	Date Range Refined (01/07/2019) - Allergan announced that the estimated PDUFA date for the company's supplemental New Drug Application (sNDA) for VRAYLAR (cariprazine) for the treatment of depressive episodes associated with bipolar I disorder (bipolar depression) in adults is expected in May 2019.
04/01/2019-06/30/2019	Assembly Biosciences, Inc.	ASMB	ABI-H0731	Hepatitis B (HBV) Treatment (Antiviral)	II	Trial Data - Top-Line Results	Phase IIa Viral Load - Top-Line Results	Assembly Biosciences announced that it expects proof-of-concept data from the Phase II study of ABI-H0731 in the second half of 2018.	Date Range Delayed (04/12/2018) - Assembly Biosciences plans to advance ABI-H0731 to Phase IIa clinical studies in summer 2018. The company anticipates results from these studies during the first half of 2019.Date Range Refined (01/07/2019) - Assembly Biosciences announced completion of patient enrollment in two Phase IIa studies with ABI-H0731 a potent core inhibitor for the treatment of HBV. The studies are ongoing and the company anticipates reporting interim results from both studies during Q2 of 2019.
01/07/2019-06/30/2019	Biogen, Inc.	BIIB	BIIB-100	Amyotrophic Lateral Sclerosis (ALS)	Preclinical	Trial Announcement - Initiation	Phase I - Trial to Start	Biogen announced that the Company plans to move KPT-350 into a Phase I study by the end of the year.	Date Range Delayed (12/31/2018) - We await an update.Date Range Refined (01/07/2019) - Biogen announced they anticipate to initiate a Phase I trial of BIIB-100 in ALS in the first half of 2019.
04/01/2019-06/30/2019	BioMarin Pharmaceutical Inc.	BMRN	Palynziq	Phenylketonuria (PKU)	Approved	Regulatory - Approval Decision (Europe)	European Approval Decision	BioMarin announced that the European Medicines Agency (EMA) has accepted BioMarin's submission of a Marketing Authorization Application (MAA) for pegvaliase a PEGylated recombinant phenylalanine ammonia lyase enzyme product for the treatment of adults with phenylketonuria (PKU) who have inadequate blood phenylalanine control (blood phenylalanine levels greater than 600 micromol/l) despite prior management with available treatment options including sapropterin. Based on an internal analysis of the centralized European approval procedure we estimate the European marketing authorization for this drug for this indication will be granted in approximately 11-17 months.	Date Range Refined (01/07/2019) - BioMarin noted during the presentation that the company is anticipating an opinion from the Committee for Medicinal Products for Human Use (CHMP) the scientific committee of the European Medicines Agency (EMA) on Palynziq (pegvaliase) Injection for the treatment of patients 16 and older with Phenylketonuria (PKU) in the first quarter of 2019. If the CHMP provides a positive opinion in the first quarter of 2019 then in the second quarter of 2019 it is possible that the European Commission (EC) could provide marketing authorization in the European Union.
01/07/2019-06/30/2019	Frequency Therapeutics, Inc.		FX-322	Hearing Loss - General	I/II	Trial Data - Top-Line Results	Phase I/II - Top-Line Results	Frequency Therapeutics announced that the first patients have been treated in a Phase I/II clinical trial to evaluate FX-322 a first-in-class drug candidate for hearing restoration from the company's Progenitor Cell Activation (PCA) regeneration platform. The Company looks forward to reporting the results from this study toward the end of 2018.	Date Range Delayed (12/31/2018) - We await an update.Date Range Delayed (01/07/2019) - Frequency anticipates data readout for the Phase I safety of FX-322 in patients with stable sensorineural hearing loss (SSHL) during the first half of 2019.
Now-06/30/2019	Onconova Therapeutics, Inc.	ONTX	Estybon (Oral)	Myelodysplastic Syndrome (MDS)	II	Trial Announcement - Initiation	Phase III Trial to Start - First Line Low Risk MDS	Onconova expects to advance to a Phase III study of 1st line oral rigosertib for lower risk MDS patients in 2014.	the Company plans to conduct a Phase III trial of oral rigosertib in transfusion-dependent lower risk MDS patients after consultation with regulatory agencies regarding rigosertib (intravenous). We await an update in the time frame above.Date Range Refined (04/02/2014) - Onconova met with the U.S. Food and Drug Administration (FDA) to discuss further development of oral rigosertib for erythropoiesis-stimulating agent (ESA) refractory transfusion-dependent lower risk myelodysplastic syndrome (MDS) patients.As a result of this dialogue Onconova has designed a pivotal Phase III protocol. Onconova intends to seek a Special Protocol Assessment (SPA) for this randomized double-blind placebo-controlled study in lower risk MDS patients who do not respond to ESAs and are dependent on packed red blood cell transfusions to alleviate their anemia. Trial size entry criteria secondary endpoints and the statistical analysis plan will be finalized during the SPA process. Onconova projects to start enrollment in the Phase III study in the second half of 2014 at multiple sites in the U.S. and Europe. Date Range Delayed (08/14/2014) - Onconova announced that a pivotal study of oral rigosertib in Lower Risk MDS is now expected to commence in the first half of 2015Date Range Delayed (12/21/2014) - Based on the time needed for completion of the prognostic genomic test and the ongoing Phase II dosing optimization study Onconova believes that a pivotal study of oral rigosertib in lower risk
Now-06/30/2019	Regeneron Pharmaceuticals, Inc.	REGN	REGN1979	Indolent Non-Hodgkin's Lymphoma (Including Follicular Lymphoma) - NHL	I	Trial Announcement - Initiation	Phase II to Start	Regeneron announced that it plans to initiate a Phase II study for REGN1979 (CD20xCD3 Antibody) in Follicular Lymphoma in 2018.	Date Range Delayed (12/01/2018) - Regeneron announced that based on data from the Phase I B-Cell Malignancies Study the Company plans to initiate in 2019 a potentially registrational Phase II trial investigating REGN1979 in R/R FL.Date Range Delayed (01/07/2019) - Regeneron expects its Phase II study of REGN1979 in follicular lymphoma to begin in the first half of 2019.
04/01/2019-06/30/2019	Spark Therapeutics, Inc.	ONCE	SPK-3006	Pompe Disease	Preclinical	Regulatory - IND Filing	IND Filing	Spark Therapeutics currently lists their Pompe disease program (SPK-GAA) in preclinical development for the treatment of Pompe disease. The Company plans to complete IND-enabling work in 2018. As such we await an IND filing update in the time frame listed above.	Date Range Delayed (10/08/2018) - Spark Therapeutics has conducted a pre-IND meeting regarding SPK-3006 with FDA. After completing an ongoing Good Laboratory Practice (GLP) toxicology and biodistribution study Spark Therapeutics will submit an IND application and Clinical Trial Application (CTA) to regulatory agencies and initiate a U.S. and EU Phase I/II clinical trial of SPK-3006 in adult patients in 2019.Date Range Refined (01/07/2019) - Spark Therapeutics will submit an IND application and Clinical Trial Application (CTA) in the second quarter of 2019 to initiate a U.S. and EU Phase I/II clinical trial of SPK-3006.
04/01/2019-06/30/2019	United Therapeutics Corporation	UTHR	Beraprost 314d	Pulmonary Arterial Hypertension (PAH) and Pulmonary Hypertension (PH)	III	Trial Data - Top-Line Results	Phase III BEAT - Top-Line Results	United Therapeutics announced that they anticipate announcing top-line results from the Phase III BEAT trial of esuberaprost for reduction in morbidity and mortality in Group 1 pulmonary arterial hypertension in 2017 or 2018.	Date Range Delayed (12/20/2018) - We await an update.Date Range Refined (01/07/2019) - United Therapeutics announced that top-line results from the Phase III BEAT study are anticipated in the second quarter of 2019.
05/01/2019-07/31/2019	Celgene Corporation	CELG	Otezla	Behçet Syndrome	NDA	Regulatory - PDUFA for sNDA/sBLA	PDUFA for sNDA - First Review	Celgene announced that in Q3 2018 a sNDA for Otezla in Behcet's disease was submitted in the U.S. Based on a standard 10-month supplemental NDA filing under PDUFA V guidelines the PDUFA decision should occur in May to July 2019.	Date Range Refined (01/07/2019) - Approval is expected by the U.S. FDA for the sNDA in Beh?et's disease with a Prescription Drug User Fee Act (PDUFA) action date of July 21 2019. As this date falls on a weekend we expect a decision the Friday before.
07/01/2019-09/30/2019	Ascendis Pharma A/S	ASND	TransCon CNP	Achondroplasia	Development Outside U.S.	Trial Announcement - Initiation	Phase II - Trial to Start	TransCon plans to initiate a Phase II program of TransCon CNP in pediatric subjects with achondroplasia in mid-2019.	Date Range Refined (01/07/2019) - Ascendis anticipates the Phase II program of TransCon CNP in pediatric subjects to initiate in the third quarter of 2019.

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Expected Date Range	Company	Symbol	Drug	Indication	Phase	Expected Catalyst	Catalyst Title	Analysis	Updated Analysis
07/01/2019-09/30/2019	BioMarin Pharmaceutical Inc.	BMRN	Valoctocogene Roxaparvovec	Hemophilia A	III	Trial Announcement - Patient Enrollment Completed	Phase III - GENEr8-1 Enrollment Completed	BioMarin plans to complete enrollment of its first Phase III study of Valoctocogene for hemophilia A by the end of 2018.	Date Range Delayed (05/22/2018) - BioMarin announced that enrollment completion in the newly amended GENEr8-1 study is expected in the first quarter of 2019.Date Range Delayed (01/07/2019) - BioMarin provided information on the company's development program for valoctocogene roxaparvovec gene therapy for severe hemophilia A. BioMarin has completed enrollment of the initial cohort of patients in its Phase III program intended to support a BLA submission through the accelerated approval pathway. The complete Phase III study is targeting enrollment of 130 patients by mid-year 2019. Enrollment completion (includes 6-month run-in; N=130) is expected in the third quarter.
07/01/2019-09/30/2019	BioMarin Pharmaceutical Inc.	BMRN	Valoctocogene Roxaparvovec	Hemophilia A	III	Trial Announcement - Patient Enrollment Completed	Phase III GENEr8-2 - Patients Enrollment Completed	BioMarin announced that the GENEr8-2 study is expected to complete enrollment one to two quarters after GENEr8-1 in 2019.	New Information (01/07/2019) - BioMarin provided information on the company's development program for valoctocogene roxaparvovec gene therapy for severe hemophilia A. BioMarin has completed enrollment of the initial cohort of patients in its Phase III program intended to support a BLA submission through the accelerated approval pathway. The complete Phase III study is targeting enrollment of 130 patients by mid-year 2019. Enrollment completion (includes 6-month run-in; N=130) is expected in the third quarter.
04/01/2019-09/30/2019	Celgene Corporation	CELG	Lisocabtagene Maraleucel	Diffuse Large B-Cell Lymphoma (DLBCL) - NHL	III	Regulatory - NDA/BLA Filing	BLA Filing	Juno plans to file a BLA for JCAR017 in relapsed / refractory DLBCL in the second half of 2018.	Date Range Delayed (11/12/2018) - Celgene announced that U.S. approval for liso-cel in RR DLBCL is now expected in mid-2020. As such we anticipate a regulatory filing in mid-2019.Date Range Delayed (01/07/2019) - A U.S. BLA submission is expected in H2:2019 for Liso-cel.
04/01/2019-09/30/2019	Edwards Lifesciences Corp.	EW	Harpoon Mitral Valve System	Cardiac Valve Surgery	Approved in Europe	Progress Update - Product Launch (Europe)	Product Launch (EU)	Edwards Lifesciences announced that it expects to begin commercialization of the Harpoon Mitral Valve System in the second half of 2018.	it received CE Mark approval for the Harpoon Device the recently acquired beating heart mitral valve repair system. The company is currently focused on integration activates and expects a mid-year 2018 launch in Europe. New Information (04/25/2018) - Edwards Lifesciencs announced that the company recently learned of complications with 3 of the 65 patients treated in the early clinical studies. While this is still early the company is looking into the root cause
07/01/2019-09/30/2019	Enanta Pharmaceuticals, Inc.	ENTA	EDP-305	Non-Alcoholic Steatohepatitis (NASH)	II	Trial Data - Top-Line Results	Phase II ARGON-1 - Top-Line Results	Enanta expects to have Phase II data in EDP-305 for the treatment of PBC and NASH in 2019.	Date Range Refined (01/07/2019) - Enrollment in the 12-week Phase IIa NASH trial is expected to conclude in the first quarter of 2019 allowing Enanta to report preliminary top line data in the third quarter of 2019.
04/01/2019-09/30/2019	Incyte Corporation	INCY	INCB54828	Biliary Tract Cancer	III	Regulatory - NDA/BLA Filing	NDA Filing	Incyte expects to submit a NDA for INCB54828 as a treatment for patients with advanced cholangiocarcinoma in 2019.	Date Range Refined (01/07/2019) - Incyte expects to submit a NDA for INCB54828 as a treatment for patients with advanced cholangiocarcinoma in mid-2019.
04/01/2019-09/30/2019	Neurocrine Biosciences, Inc.	NBIX	NBI-74788	Congenital Adrenal Hyperplasia (CAH)	II	Trial Announcement - Initiation	Phase IIa - Pediatric Trial Initiation	Pending positive results from the Phase II study of NBI-74788 in adults with Congenital Adrenal Hyperplasia (CAH) a Phase II study in pediatric patients is planned to initiate in 2H 2018.	Date Range Delayed (12/31/2018) - Neurocrine Biosciences has not yet provided an update on the pediatric clinical study. We have contacted the Company and await an update in the time frame above.Date Range Delayed (01/07/2019) - Neurocrine expects the Phase IIa initiation for NBI-74788 in CAH (pediatric) in Q2/Q3 2019.
07/01/2019-09/30/2019	Spark Therapeutics, Inc.	ONCE	SPK-TPP1	Neuronal Ceroid Lipofuscinosis (NCL)	Preclinical	Regulatory - IND Filing	IND Filing	Spark Therapeutics expects an IND filing for SPK-TPP1 in the second half of 2016 with initial efficacy data in the first half of 2017.	Date Range Delayed (11/17/2016) - Spark announced that further optimization of the trial design for SPK-TPP1 is warranted and as such are no longer are targeting a trial start this year. We await an update in the time frame above.Date Range Delayed (03/08/2017) - SPK-TPP1 is currently listed in preclinical development in Spark's pipeline. We await an update in the time frame above.Date Range Delayed (06/28/2017) - We continue to await an update.Date Range Delayed (08/24/2017) - We continue to await an update.Date Range Delayed (11/20/2017) - We continue to await an update.Date Range Delayed (02/23/2018) - SPK-TPP1 is currently listed in preclinical development in Spark's pipeline. We await an update in the time frame above.Date Range Delayed (05/18/2018) - We continue to await an update.Date Range Delayed (09/28/2018) - SPK-TPP1 for the treatment of CLN2 disease (neuronal ceroid lipofuscinosis (NCL)) caused by TPP1 deficiency remains a preclinical program. No timing for an IND was provided. We continue to await an update.Date Range Delayed (01/07/2019) - Spark Therapeutics expects to complete IND-enabling work on SPK-1001 for CLN2 disease in the first half of 2019. As such we await an update on the filing in the time frame above.
Now-12/31/2019	Akcea Therapeutics, Inc.	AKCA	AKCEA-APO(a)-LRx	Cardiovascular Disease	II	Trial Announcement - Initiation	Phase III Trials to Start	ISIS announced plans to initiate two Phase III studies in late 2017/ early 2018 for ISIS-APO(a)-L_{RX}. The first study will be in recurrent cardiovascular events with very high Lp(a) and the second is in calcific aortic valve stenosis with high Lp(a).	New Information (01/06/2017) - Ionis Pharmaceuticals and Akcea Therapeutics announced an exclusive worldwide option and collaboration agreement with Novartis to develop and commercialize AKCEA-APO(a)-LRx and AKCEA-APOCIII-LRx. Ionis and Akcea plan to conduct a Phase II dose-ranging study for each drug to choose the optimal dose and evaluate alternative dose schedules such as monthly dosing for the Phase III study. For each drug upon option exercise Novartis will pay Ionis and Akcea a $150 million license fee will initiate a global Phase III cardiovascular outcome study in a high-risk population and will be responsible for worldwide development and commercialization activities.Date Range Delayed (03/15/2018) - Under Akcea's agreement with Novartis after it completes Phase II development of AKCEA-APO(a)-
10/01/2019-12/31/2019	Alnylam Pharmaceuticals, Inc.	ALNY	Lumasiran	Hyperoxaluria	III	Trial Data - Top-Line Results	Phase III ILLUMINATE-A - Top-Line Results	Alnylam Pharmaceuticals announced that the Company is on track to start the Phase III study in mid-2018 and is now guiding that it expects to report top-line results in 2019.	Date Range Refined (10/04/2018) - Alnylam expects to report topline results from ILLUMINATE-A study of Lumasiran in late 2019 and if positive submit filings for regulatory approval starting in early 2020.Date Range Delayed (01/07/2019) - Alnylam announced at the 37th Annual J.P. Morgan Healthcare Investor Conference that the company expects to report top-line results from the Phase III - ILLUMINATE-A on lumasiran for hyperoxaluria in late 2019.
07/01/2019-12/31/2019	AMAG Pharmaceuticals, Inc.	AMAG	Bremelanotide	Female Sexual Arousal Disorder	NDA	Progress Update - Product Launch (U.S.)	Product Launch (U.S.)	AMAG anticipates FDA action and commercial launch of bremelanotide for the treatment of hypoactive sexual desire disorder (HSDD) in early 2019.	Date Range Delayed (01/07/2019) - AMAG and Palatin the North American licensee of Vyleesi (bremelanotide) announced an update on the regulatory development of Vyleesi noting that the U.S. Food and Drug Administration has extended the Prescription Drug User Fee Act (PDUFA) date by three months to June 23 2019. We await an update on the commercialization of Vyleesi in the time frame above.
Now-12/31/2019	Celgene Corporation	CELG	Fedratinib	Myelofibrosis (MF)	III	Regulatory - MAA Submission (Europe)	MAA Submission	Celgene announced that the EU MAA submission for fedratinib is planned in 2019.	Date Range Refined (01/07/2019) - Celgene announced that the EU MAA submission for fedratinib is planned for the first half of 2019.

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Expected Date Range	Company	Symbol	Drug	Indication	Phase	Expected Catalyst	Catalyst Title	Analysis	Updated Analysis
01/07/2019-12/31/2019	DexCom, Inc.	DXCM	Dexcom G7 Continuous Glucose Monitoring System	Diabetes Mellitus, Type I	IDE	Regulatory - PMA Filing	PMA Filing	Dexcom announced it will file a PMA application for its Verily G1 device subsequently after filing its G6 CGM device with the FDA. The company expects to submit a PMA application for the G6 CGM to the FDA by Q3 of 2017. We await an update in the time frame above for an update on the application submission for the Verily CGM device.	Date Range Delayed (11/07/2017) - Dexcom announced that it looks to complete development of the first-generation CGM system with Verily in the first half of 2018. The company did not provide any timeline estimates regarding FDA filing or approval therefore we look for an update on the PMA filing in the time frame above. Date Range Expedited (01/12/2018) - Dexcom announced that it expects to launch the Verily System in late 2018 or early 2019. Given this information we anticipate an update regarding the PMA filing in the time frame listed above.Date Range Delayed (08/30/2018) - DexCom announced that it is currently in the validation and verification phase of development with the Verily System. The Company did not provide an updated development timeline therefore we anticipate an update in the time frame listed above.Date Range Delayed (12/31/2018) - DexCom has not yet provided an update on the development status of the Verily System. We have contacted the Company and await an update in the time frame above.New Information (01/07/2019) - Dexcom announced that the company's time frame for the G7 CGM is an initial launch in late 2020 with a full roll-out in 2021. Given this information we look for an update on the approval filing decision in the time frame above.
07/01/2019-12/31/2019	Enanta Pharmaceuticals, Inc.	ENTA	EDP 514	Hepatitis B (HBV) Treatment (Antiviral)	Preclinical	Trial Announcement - Initiation	Clinical Trials to Start	Enatna announced that the Company is currently studying a core inhibitor for the treatment of hepatitis B virus and a non-fusion inhibitor for the treatment of RSV. Both compounds are in preclinical development and the Company expects to advance one of the two product candidates to Phase I clinical studies in 2017.	Date Range Delayed (12/12/2017) - Enanta announced the Company continues to make progress in discovering characterizing and seeking patent protection for new core inhibitors of HBV with the goal of identifying a development candidate in 2018. As such we await an update on the start of clinical trials in 2018. New Information (01/09/2018) - Enanta's current research efforts in HBV are focused on core inhibitors with the aim of developing a functional cure. Preclinical lead optimization continues to progress with the goal of identifying a development candidate in 2018. Date Range Delayed (12/31/2018) - We await an update.Date Range Delayed (01/07/2019) - A Phase I study of EDP-514 is planned to begin in the second half of 2019. The study will evaluate single andmultiple doses of drug in healthy volunteers and will incorporate a Phase Ib arm in patients with chronic HBVinfection.
01/07/2019-12/31/2019	Enzyvant Therapeutics, Inc.		RVT-801	Farber Disease	Preclinical	Trial Announcement - Initiation	Clinical Trial Initiation	Enzyvant is conducting preclinical studies for RVT-801 a recombinant form of human acid ceramidase (rhAC) to enable a clinical trial of rhAC as an enzyme replacement therapy in patients with Farber disease. Subject to regulatory review the company intends to initiate a clinical trial of RVT-801 in 2018.	Date Range Delayed (12/31/2018) - We await an update.Date Range Delayed (01/07/2019) - Roivant Sciences expects the initiation of a clinical trial for the treatment of Farber disease in 2019.
Now-12/31/2019	Epizyme, Inc.	EPZM	Tazemetostat	Indolent Non-Hodgkin's Lymphoma (Including Follicular Lymphoma) - NHL	II	Trial Announcement - Initiation	Phase II - Combination Trial to Start	Epizyme announced that a Phase II study of Tazemetostat in combination with an undisclosed product for the treatment of relapsed/refratrory follicular lymphoma is expected to begin in 2017.	Date Range Delayed (11/16/2017) - Epizyme plans to begin a combination trial of tazemetostat in patients with FL in 2018.Date Range Delayed (12/31/2018) - We await an update.Date Range Delayed (01/07/2019) - Epizyme reported that it plans to conduct a combination study that would compare the chemo-free combination of rituximab and Revlimid (R2) with tazemetostat versus R2 with placebo in patients with relapsed or refractory FL in 2019.
Now-12/31/2019	Genmab A/S	GEN:DC	Tisotumab Vedotin	Cervical Cancer	II	Trial Announcement - Initiation	Phase II Combination Regimen - Trial to Start	Seattle Genetics anticipates initiating a Phase II trial of TV as part of a combination regimen for first-line cervical cancer in 2018.	Date Range Delayed (12/26/2018) - We await an update.Date Range Refined (01/07/2019) - Seattle Genetics announced they anticipate enrollment of innovaTV 205 to begin in 2019.
09/01/2019-12/31/2019	H. Lundbeck A/S	HLUKF	Foliglurax	Parkinson's Disease (PD)	I	Trial Data - Top-Line Results	Phase II AMBLED - Top-Line Results	Prexton announces the launch of Phase II clinical testing of its investigational drug candidate Foliglurax in Parkinson's disease (PD). Data is expected in 2019.	Date Range Refined (03/16/2018) - Lundbeck announced data from the Phase II study of foliglurax for the treatment of Parkinson's disease is expected in the first half of 2019.Date Range Delayed (01/07/2019) - Lundbeck anticipates headline results (PoC) for foliglurax in Parkinson's Disease in the second half of 2019.
Now-12/31/2019	Incyte Corporation	INCY	Itacitinib	Graft vs. Host Disease (GVHD)	III	Trial Data - Top-Line Results	Phase III GRAVITAS - Top-Line Results	Incyte expects data in 2019 for the Phase III GRAVITAS study of Itacitinib in steroid naive 1st line acute GVHD.	Date Range Refined (01/07/2019) - Incyte expects top-line data from the Phase III GRAVITAS 301 study of Itacitinib in H2 2019.
01/07/2019-12/31/2019	Jazz Pharmaceuticals plc	JAZZ	Defitelio	Cardiovascular Disease	Preclinical	Trial Announcement - Initiation	Phase II - TA-TMA Study to Start	Jazz plans to activate a site for the pivotal Phase II study of Debirotide for the treatment of Transplant-Associated Thrombotic Microangiopathy (TA-TMA) in the fourth quarter of 2018.	Date Range Delayed (12/31/2018) - We await an update.Date Range Delayed (01/03/2019) - Jazz Pharmaceuticals has not provided an update on the Phase II study we continue to await an update. New Information (01/07/2019) - Jazz plans to activate study sites for the pivotal Phase II study of Debirotide for the treatment of Transplant-Associated Thrombotic Microangiopathy (TA-TMA) in 2019.
01/07/2019-12/31/2019	Kiniksa Pharmaceuticals Corporation	KNSA	KPL-716	Atopic Dermatitis (Eczema)	I	Trial Announcement - Initiation	Phase IIa/b - Trial to Start	Kiniksa announced the Company plans to start Phase II studies of KPL-716 in 2018.	Date Range Delayed (12/31/2018) - We await an update.Date Range Delayed (01/07/2019) - Kiniksa plans to initiate a Phase IIa/b study of KPL-716 for the treatment of prurigo nodularis in 2019.
07/01/2019-12/31/2019	Kiniksa Pharmaceuticals Corporation	KNSA	KPL-045	Autoimmune Disorders	Preclinical	Regulatory - IND Filing	IND Filing	Kiniksa announced they are planning IND-enabling studies of KPL-045 in T-cell dependent B-cell mediated diseases and expect to file an IND with the FDA for this program in 2019.	Date Range Refined (01/07/2019) - Kiniksa plans to file an IND for KPL-045 in the second half of 2019.
07/01/2019-12/31/2019	Kiniksa Pharmaceuticals Corporation	KNSA	KPL-404	Autoimmune Disorders	Preclinical	Regulatory - IND Filing	IND Filing	Kiniksa announced they are planning IND-enabling studies of KPL-404 in T-cell dependent B-cell mediated diseases and expect to file an IND with the FDA for this program in 2019.	Date Range Refined (01/07/2019) - Kiniksa plans to file an IND for KPL-404 in the second half of 2019.

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Expected Date Range	Company	Symbol	Drug	Indication	Phase	Expected Catalyst	Catalyst Title	Analysis	Updated Analysis
07/01/2019-12/31/2019	Mallinckrodt plc	MNK	Terlipressin	Hepatorenal Syndrome (HRS)	III	Trial Data - Top-Line Results	Phase III CONFIRM - Interim Analysis	Mallinckrodt announced that the Company has planned an interim analysis for the Phase III CONFIRM study of Terlipressin. As such we await an update on results from this study in the time frame above.	announced it has enrolled the 75th subject in the company's ongoing Phase III pivotal trial CONFIRM to evaluate the efficacy and safety of terlipressin (for injection) in subjects with Hepatorenal Syndrome (HRS) type 1. An interim analysis of trial data to assess the safety and efficacy of terlipressin in study subjects will be completed when 150 subjects are enrolled and this also marks the halfway point toward that milestone. We continue to await an update.Date Range Delayed (10/04/2017) - Mallinckrodt expects to report top-line results from the Phase III study of Terlipressin in Hepatorenal Syndrome (HRS) type-1 during the first half of 2020.Date Range Expedited (01/08/2018) - Mallinckrodt expects to report an interim analysis from the Phase III study of Terlipressin in Hepatorenal Syndrome (HRS) type-1 during the first half of 2018.Date Range Delayed (06/22/2018) - We continue to await an update in the time frame listed above.Date Range Delayed (09/27/2018) - We continue to await an update in the time frame listed above.Date Range Delayed (11/27/2018) - Mallinckrodt announced that its Phase III trial of terlipressin for the treatment of hepatorenal syndrome type 1 is over 75% enrolled and the company anticipates completing this program in the second half of 2019. We await an update in the time frame above.New Information (01/07/2019) - Mallinckrodt announced that results for its Phase III trial of terlipressin for the treatment of hepato-renal syndrome
07/01/2019-12/31/2019	Mallinckrodt plc	MNK	StrataGraft Skin Tissue	Burn Injury	III	Trial Data - Top-Line Results	Phase III - Top-Line Results	Data from the Phase III study of stratagraft to treat deep partial thickness components is expected in the first half of 2020.	Date Range Expedited (01/07/2019) - Mallinckrodt expects to announce top-line data from the Phase III study of StrataGraft in deep partial thickness severe burns in the second half of 2019.
07/01/2019-12/31/2019	Neurocrine Biosciences, Inc.	NBIX	NBI-74788	Congenital Adrenal Hyperplasia (CAH)	II	Trial Announcement - Initiation	Phase III - Adult Trial Initiation	Pending positive results from the Phase II study of NBI-74788 in adults with Congenital Adrenal Hyperplasia (CAH) a Phase III study in adults is planned to initiate in 2H 2018.	Date Range Delayed (12/31/2018) - Neurocrine Biosciences has not yet provided an update on the Phase III study. We have contacted the Company and await an update in the time frame above.Date Range Delayed (01/07/2019) - Pending a U.S. Food and Drug Administration (FDA) discussion in the second quarter Neurocrine expects a Phase III study in adults to initiate in 2H 2019.
01/07/2019-12/31/2019	Regeneron Pharmaceuticals, Inc.	REGN	Dupixent	Chronic Obstructive Pulmonary Disease (COPD)	I	Trial Announcement - Initiation	Phase III Study to Start	Regeneron announced that pivotal Phase III studies are planned in 2018 evaluating the use of dupilumab in chronic obstructive pulmonarydisease (COPD).	Date Range Refined (02/07/2018) - Sanofi announced the company expects to start a Phase III trial with dupilumab for the treatment of COPD in the fourth quarter of 2018.Date Range Delayed (12/31/2018) - We await an update.Date Range Delayed (01/07/2019) - Regeneron plans to initiate the Phase II/III study of Dupixent for COPD in 2019.
01/07/2019-12/31/2019	Regeneron Pharmaceuticals, Inc.	REGN	REGN-3918	Paroxysmal Nocturnal Hemoglobinuria (PNH)	I	Trial Announcement - Initiation	Phase II to Start	Regeneron expects to report full data from its Phase I study of REGN3918 in paroxysmal nocturnal hemoglobinuria (PNH) in the second half of 2018 and plans to initiate a Phase 2 study in PNH in early 2019.	Date Range Delayed (01/07/2019) - Regeneron plans to initiate the Phase II study of Pozelimab in PNH in 2019.
01/07/2019-12/31/2019	Sarepta Therapeutics, Inc.	SRPT	Microdystrophin Gene Therapy Program (NCH)	Duchenne Muscular Dystrophy (DMD)	I/II	Trial Announcement - Initiation	Pivotal Study to Start	Sarepta anticipates dosing patients in the pivotal trial for the micro dystrophin program by year-end 2018.	Date Range Delayed (12/28/2018) - We await an update.Date Range Delayed (01/07/2019) - Sarepta plans to conduct a confirmatory trial of its micro-dystrophin program with commercial material in 2019.
04/01/2019-03/31/2020	Daiichi Sankyo Co., Ltd.	DSKYF	DS-8201	Breast Cancer	III	Trial Announcement - Initiation	Phase Ib w/Keytruda (Breast/NSCLC) - Trial to Start	Daiichi Sankyo announced that a Phase I/II study of DS-8201 in combination with an Immuno-Oncology (IO) drug in patients with HER2+ breast and NSCLC is expected in the third quarter of fiscal year 2018.	New Information (09/20/2018) - Daiichi Sankyo announced it has entered into a clinical trial collaboration agreement with a subsidiary of Merck to evaluate the combination of Daiichi Sankyo's DS-8201 and KEYTRUDA (pembrolizumab) in HER2 expressing advanced/metastatic breast and HER2 expressing or HER2 mutant non-small cell lung cancers (NSCLC). We continue to await the start of this trial in the time frame above. Date Range Delayed (12/31/2018) - We await an update.Date Range Delayed (01/07/2019) - Daiichi Sankyo expects to initiate a Phase Ib trial of DS-8201 in combination with Keytruda for the treatment of breast cancer and NSCLC in the company's fiscal year 2019.
04/01/2019-03/31/2020	Daiichi Sankyo Co., Ltd.	DSKYF	DS-8201	Non-Small Cell Lung Cancer (NSCLC)	II	Trial Announcement - Initiation	Phase Ib w/Keytruda (Breast/NSCLC) - Trial to Start	Daiichi Sankyo announced it has entered into a clinical trial collaboration agreement with a subsidiary of Merck to evaluate the combination of Daiichi Sankyo's DS-8201 and KEYTRUDA (pembrolizumab) in HER2 expressing advanced/metastatic breast and HER2 expressing or HER2 mutant non-small cell lung cancers (NSCLC). We await the start of this trial in the time frame above.	Date Range Delayed (12/31/2018) - We await an update.Date Range Delayed (01/07/2019) - Daiichi Sankyo expects to initiate a Phase Ib trial of DS-8201 in combination with Keytruda for the treatment of breast cancer and NSCLC in the company's fiscal year 2019.
04/01/2019-03/31/2020	Daiichi Sankyo Co., Ltd.	DSKYF	DS-8201	Solid Tumors	I	Trial Announcement - Initiation	Phase Ib w/Avelumab to Start	Daiichi Sankyo announced that it has entered into a clinical trial collaboration agreement with Merck KGaA and Pfizer to evaluate the combination of [fam-] trastuzumab deruxtecan (DS-8201) an investigational HER2 targeting antibody drug conjugate (ADC) in combination with the checkpoint inhibitor avelumab and/or an investigational Merck KGaA Darmstadt Germany DNA damage response (DDR) inhibitor in patients with HER2 expressing or mutated solid tumors. We await an update through the first half of 2019.	Date Range Delayed (01/07/2019) - Daiichi Sankyo expects to initiate a Phase Ib trial of DS-8201 in combination with avelumab for the treatment of solid tumors in the company's fiscal year 2019.
07/01/2019-04/30/2020	Roivant Sciences, Inc.		Tapinarof	Atopic Dermatitis (Eczema)	IIb	Trial Data - Top-Line Results	Phase III - Top-Line Results	GlaxoSmithKline announced that Phase III data of GSK2894512 for the treatment of atopic dermatitis and psoriasis are expected in the first half of 2019.	Date Range Delayed (01/07/2019) - Roivant Sciences expects the initiation of two Phase III trials of tapinarof for psoriasis in 2019. We await an updated timeline on the data from these studies.
07/01/2019-04/30/2020	Roivant Sciences, Inc.		Tapinarof	Psoriasis	IIb	Trial Data - Top-Line Results	Phase III - Top-Line Results	GlaxoSmithKline announced that Phase III data of GSK2894512 for the treatment of atopic dermatitis and psoriasis are expected in the first half of 2019.	Date Range Delayed (01/07/2019) - Roivant Sciences expects the initiation of two Phase III trials of tapinarof for psoriasis in 2019. We await an updated timeline on the data from these studies.
01/01/2020-06/30/2020	Karyopharm Therapeutics	KPTI	Selinexor	Diffuse Large B-Cell Lymphoma (DLBCL) - NHL	IIb	Progress Update - Product Launch (U.S.)	Product Launch (U.S.)	Karyopharm announces a product launch in the United States for Selinexor for the treatment of Diffuse Large B-Cell Lymphoma (DLBCL) in the end of 2019.	Date Range Delayed (01/07/2019) - Karyopharm now anticipates launch in the US in H1 2020.
01/01/2020-06/30/2020	Mallinckrodt plc	MNK	H.P. Acthar Gel	Systemic Lupus Erythematosus (SLE)	Approved	Trial Data - Top-Line Results	Phase IV - Top-Line Results	Mallinckrodt announced that it expects data from the Phase IV study of HP Acthar Gel in SLE in the second half of 2019.	Date Range Delayed (01/07/2019) - Mallinckrodt announced that data from the Phase IV study of H.P. Acthar Gel for the treatment of SLE are expected in the first half of 2020.
01/01/2020-06/30/2020	Mallinckrodt plc	MNK	H.P. Acthar Gel	Multiple Sclerosis (MS)	Approved	Trial Data - Top-Line Results	Phase IV - Top-Line Results	Mallinckrodt announced that data from the Phase IV study of HP Acthar Gel for the treatment of multiple sclerosis is expected in the second half of 2018.	Date Range Delayed (12/19/2018) - Mallinckrodt has not provided an update on the release of the results from the Phase IV study of H.P. Acthar Gel for Multiple Sclerosis we continue to await an update. Date Range Delayed (01/07/2019) - Mallinckrodt announced that data from the Phase IV study of H.P. Acthar Gel for the treatment of multiple sclerosis are expected in the first half of 2020.
01/01/2020-06/30/2020	Rhythm Pharmaceuticals, Inc.	RYTM	Relamorelin	Diabetic Gastroparesis	III	Trial Data - Top-Line Results	Phase III - Top-Line Results	Allergan announced that topline data from the Phase III study of relamorelin in diabetic gastroparesis is expected in 2019.	Date Range Delayed (08/03/2017) - Allergan lists that top-line data from the Phase III study of relamorelin for the treatment of diabetic gastroparesis are expected in 2020.Date Range Refined (01/07/2019) - Allergan reported that top-line data from the Phase III study of relamorelin for the treatment of diabetic gastroparesis are expected in the first half of 2020.
01/01/2020-12/31/2020	Agios Pharmaceuticals, Inc.	AGIO	Tibsovo	Acute Myelogenous Leukemia (AML)	Approved	Trial Announcement - Patient Enrollment Completed	Phase III - AGILE - Patient Enrollment Completed	Agios expects to complete enrollement for the Phase III AGILE study of ivosidenib and VIDAZA in newly diagnosed AML patients with an IDH1 mutation who are not candidates for intensive chemotherapy in 2021.	Date Range Expedited (01/07/2019) - Agios expects to complete enrollment of its Phase III AGILE study in 2020.

Biomedtracker/Meddevicetracker JPM Updated Catalysts - Day 1

Expected Date Range	Company	Symbol	Drug	Indication	Phase	Expected Catalyst	Catalyst Title	Analysis	Updated Analysis
01/01/2020-12/31/2020	AstraZeneca PLC	AZN	AZD4635	Solid Tumors	I	Trial Announcement - Trial Completion	Phase I w/Durvalumab - Trial Complete	The Phase I trial of AZD4635 for solid tumors sponsored by AstraZeneca is expected to complete in the second half of 2019.	Date Range Delayed (01/07/2019) - Sosei announced that the company plans for the Phase I study of AZD4635 as a monotherapy or in combination with durvalumab for the treatment of solid tumors to complete in 2020.
Now-12/31/2020	DexCom, Inc.	DXCM	Dexcom G7 Continuous Glucose Monitoring System	Diabetes Mellitus, Type I	IDE	Regulatory - PMA Approval Decision	PMA Approval Decision	DexCom announced it has received IDE approval for its Verily CGM product. It plans to launch the product in 2018. Given this time line we expect the company to receive FDA approval in the time frame above.	will file a PMA application for its Verily G1 device subsequently after filing its G6 CGM device with the FDA. The company expects to submit a PMA application for its G6 CGM to the FDA by Q3 of 2017. The company also announced it plans to commercialize its Verily CGM product by the end of 2018. Given this information we expect the device to receive approval in the time frame above.Date Range Delayed (11/07/2017) - Dexcom announced that it looks to complete development of the first-generation CGM system with Verily in the first half of 2018. The company did not provide any timeline estimates regarding FDA filing or approval therefore we look for an update on the PMA approval in the time frame above.Date Range Expedited (01/12/2018) - Dexcom announced that it expects to launch the Verily System in late 2018 or early 2019. Given this information we anticipate an update regarding the PMA approval in the time frame listed above.Date Range Delayed (08/30/2018) - DexCom announced that it is currently in the validation and verification phase of development with the Verily System. The Company did not provide an updated development timeline therefore we anticipate an update in the time frame listed above.Date Range Delayed (12/31/2018) - DexCom has not yet provided an update on the development status of the Verily System. We have contacted the Company and await an update in the time frame above.Date Range Delayed (01/07/2019) - Dexcom
01/01/2020-12/31/2020	Incyte Corporation	INCY	INCB50465	Indolent Non-Hodgkin's Lymphoma (Including Follicular Lymphoma) - NHL	II	Trial Data - Top-Line Results	Phase II CITADEL-203 - Top-Line Results	Incyte expects data from the Phase II CITADEL-203 evaluating INCB50465 will be available in 2019.	Date Range Delayed (01/07/2019) - Incyte announced initial data from parsaclisib trials in follicular mantle celland marginal zone lymphoma are expected in 2020.
01/01/2020-12/31/2020	Incyte Corporation	INCY	INCB50465	Marginal Zone Lymphoma - NHL	II	Trial Data - Top-Line Results	Phase II CITADEL-204 - Top-Line Results	Incyte expects initial data from the CITADEL-204 evaluating INCB50465 to be available in 2019.	Date Range Delayed (01/07/2019) - Incyte announced initial data from parsaclisib trials in follicular mantle cell and marginal zone lymphoma are expected in 2020.
01/01/2020-12/31/2020	Incyte Corporation	INCY	INCB50465	Mantle Cell Lymphoma - NHL	II	Trial Data - Top-Line Results	Phase II CITADEL-205 - Top-Line Results	Incyte expects initial data from the Phase II CITADEL-205 study evaluating INCB50465 to be available in 2019.	Date Range Delayed (01/07/2019) - Incyte announced initial data from parsaclisib trials in follicular mantle cell and marginal zone lymphoma are expected in 2020.
01/01/2020-12/31/2020	SpringWorks Therapeutics, LLC		Nirogacestat	Solid Tumors	II	Trial Data - Top-Line Results	Phase III - Top-Line Results	Springworks announced they anticipate a top-line data readout for their Phase III trial of nirogacestat for the treatment of sarcoma in early 2021.	Date Range Expedited (01/07/2019) - SpringWorks anticipates an interim analysis for the Phase III study of Nirogacestat for the treatment of desmoid tumor to take place in 2020.
01/01/2021-06/30/2021	Mallinckrodt plc	MNK	H.P. Acthar Gel	Amyotrophic Lateral Sclerosis (ALS)	II	Trial Data - Top-Line Results	Phase IIb - Top-Line Results	Data from the Phase II study of H.P. Acthar Gel in subjects with ALS is expected in the first half of 2020.	Date Range Delayed (01/07/2019) - Data from the Phase II study of H.P. Acthar Gel in subjects with ALS are expected in the first half of 2021.
10/01/2020-12/31/2021	DexCom, Inc.	DXCM	Dexcom G7 Continuous Glucose Monitoring System	Diabetes Mellitus, Type I	IDE	Progress Update - Product Launch (U.S.)	Product Launch - US	Dexcom announced that it plans to commercialize its Verily CGM product by the end of 2018.	Date Range Delayed (11/07/2017) - Dexcom announced that it looks to complete development of the first-generation CGM system with Verily in the first half of 2018. The company did not provide any timeline estimates regarding FDA filing or approval therefore we look for an update on the product launch in the time frame above. Date Range Refined (01/12/2018) - Dexcom announced that it expects to launch the Verily System in late 2018 or early 2019.Date Range Delayed (01/07/2019) - Dexcom announced that the company's time frame for the G7 CGM is an initial launch in late 2020 with a full roll-out in 2021.
01/01/2022-06/30/2022	Mallinckrodt plc	MNK	StrataGraft Skin Tissue	Burn Injury	III	Trial Data - Top-Line Results	Phase II - STRATA2014 - Top-Line Results	Mallinckrodt expects to announce top-line data from the Phase II study of StrataGraft in full-thickness severe burns in the second half of 2019.	Date Range Delayed (01/07/2019) - Mallinckrodt expects to announce top-line data from the Phase II study of StrataGraft in full-thickness severe burns in the first half of 2022.
07/01/2022-12/31/2022	Marathon Pharmaceuticals LLC		Synacthen Depot	Duchenne Muscular Dystrophy (DMD)	II	Trial Data - Top-Line Results	Phase II - Top-Line Results	Data from the Phase II study of MNK-1411 to treat DMD is expected in the first half of 2021.	Date Range Delayed (01/07/2019) - Mallinckrodt announced results from a Phase II study of MNK-1411 for the treatment of DMD are anticipated in the second half of 2022.