CITELINE COMMERCIAL

#JPM2025 TRACKED & UNPACKED

WHAT'S THE BIG D£AL?

#JPM2025 POST-EVENT REPORT

Go beyond the headlines with our expert analysis of the biggest news from the J.P. Morgan Healthcare Conference.

Sponsored by ICON BIOTECH

Summary

The 43rd annual J.P. Morgan Healthcare Conference (JPM) was held in San Francisco, CA over Jan. 13–16, 2025. This report contains presentation highlights from a selection of companies from the conference in a single destination. A complete list of events and catalysts that were announced or updated is included as a supplement to the report.

About the Author

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

Disclaimer

Table of Contents

Mega Cap

AbbVie

AbbVie's CEO, Rob Michael, who has been with the company for over a year, began the session by saying he is very pleased with the company's current position. At the fireside chat at the J.P. Morgan Healthcare Conference, Rob stated that the company is well ahead in terms of earnings and is focused on driving the growth platform. This growth platform comprises immunology, oncology, neuroscience, aesthetics, and eye care and he estimates that it would put the company in a stable financial position for the next eight years. This segment alone generates over 80% of revenue and has a 17% growth estimated for 2024. Despite Humira's patent loss, this will allow AbbVie to grow by 4%. The company is also focusing on developing and expanding its pipeline, which will continue to generate growth in the following years.

In the future, Rob expects continued momentum from Skyrizi and Rinvoq, particularly in IBD, and growth in neuroscience with products like Vraylar, Botox, Ubrelvy, and Qulipta. The company also anticipates the approval of the GCA indication for Rinvoq and the accelerated approval of Teliso-V in lung cancer. Growth will be partially offset by a further step down in Humira revenue and the impact of the Part D benefit redesign, which is expected to have a roughly 3% impact on the top line. Despite these offsets, the company expects to achieve mid-single-digit topline growth and a similar EPS growth and is confident in delivering high single-digit growth through 2029. Net interest expenses are expected to increase due to recent acquisitions. AbbVie has signed 20 deals, with most focusing on early-stage opportunities, primarily in immunology, oncology, and neuroscience.

In the field of immunology, AbbVie believes there is room for innovation in the IBD market, despite having strong products like Skyrizi and Rinvoq. Humira sales have declined due to the loss of patents and biosimilar erosion. However, the company has seen some patients switch from Humira to Skyrizi and Rinvoq, and they anticipate a similar trend when payers try to switch patients from Stelara driving further growth. Skyrizi and Rinvoq are showing tremendous momentum, particularly in IBD, with strong capture rates in Crohn's and UC. The company is capturing over 50% of the IBD market when combining both brands. The company sees IL-23s as a growing category and feels confident about its position with Skyrizi. Rob added that they recognize Tremfya as a strong competitor but are optimistic about Skyrizi's more substantial portfolio. In addition to IL-23s, AbbVie anticipates that TL1As will enter the market later in the decade but does not expect them to disrupt the standards set by their current brands and is focusing on developing combination therapies, particularly combining TL1A with Skyrizi to shift the standard of care.

The company is also looking into oral options, like the platform from Nimble Therapeutics, which it plans to acquire. Nimble's lead asset, an investigational oral peptide, has high potency and a long half-life, making it suitable for combination therapies. Besides expanding the market in approved indications, AbbVie plans to extend its reach in new indications. Rinvoq is being pursued in GCA, alopecia areata, vitiligo, lupus, and hidradenitis suppurativa, which are expected to contribute over a billion in peak sales.

The company is heavily invested in neuroscience, its second largest therapeutic area, and is experiencing strong growth. They explore various novel approaches, including psychoplastogens, next-generation antibodies, and new treatments for Parkinson's and essential tremor. The company is focused on early-stage investments in neuroscience as they have a clear path to growth over the next eight years and are building a pipeline to drive growth beyond that timeframe.

Over in aesthetics, the company views it as a highly underpenetrated market but has seen challenges in consumer sentiment, particularly in the filler space. AbbVie has seen toxins grow, but fillers have been more difficult because they are higher priced and can be deferred. The company has continued innovation, with new indications in Asia and a short-acting toxin that they believe can drive market growth and share and aim for a long-term target of 9 billion in revenue for the aesthetics business.

Moving forward, AbbVie's priority remains investing in early-stage assets to drive growth in the next decade. The company has a strong M&A track record with acquisitions like Skyrizi and Allergan. The company is developing various oncology assets, including ADCs targeting CMAT, and is exploring tumor-agnostic approaches while focusing on tri-specifics, multi-specifics, T cell engagers, and CAR-T opportunities. AbbVie is also developing assets in hematologic malignancies and multiple myeloma. Overall, the company is well positioned with a diversified portfolio, a strong commitment to R&D, and a strategic focus on both immediate growth and long-term pipeline development, suggesting confidence in its ability to deliver sustained growth in the coming years.

AstraZeneca

AstraZeneca CFO Aradhana Sarin started with an overview of AstraZeneca describing its strategic focus on five therapy areas: (i) oncology, (ii) cardiovascular, renal and metabolism, (iii) respiratory and immunology, (IV) vaccine and immune therapies and (V) rare diseases. Sarin noted that in the first nine months of 2024, total revenues were $39.2bn, a 19% year over year increase. However, during the Q&A, Sarin stated that AstraZeneca is unlikely to see growth in the high teens in 2025. Some factors slowing growth include Farxiga value-based purchasing, loss of exclusivity for Soliris, pricing pressure due to the inflation reduction act as well as some downturn in China due to

ongoing investigations by Chinese regulators into alleged illegal drug imports, data breaches, and potential health insurance fraud. The investigation has led to the arrest of AstraZeneca's China president as well as other employees and is expected to impact the company's China sales which account for approximately 13% of total revenue. Sarin also noted that AstraZeneca reports revenues in US dollars and that with the significant strengthening of the dollar, this will affect revenues in 2025 since 60% of revenues are generated outside of the US.

In 2024, AstraZeneca had nine positive Phase III trials and cumulatively, these opportunities represent more than $5bn in incremental peak sales revenue. AstraZeneca expects to launch 20 medicines by 2030 and deliver $80bn in risk adjusted total revenue by the end of the decade. Towards that goal, AstraZeneca expects Phase III data in 2025 that could lead to lead to label expansions including Fasenra for COPD and Breztri for severe asthma as well as the first Phase III data for seven new therapies. Sarin gave an overview of some of the Phase III data expected in 2025 starting with Enhertu for HER2+ breast cancer.

In 2024, the HER2 targeted antibody-drug conjugate Enhertu reported positive results from DESTINY-Breast06 during the ASCO plenary session. The trial supported regulatory submissions to the FDA and EMA for chemotherapy naïve, unresectable or metastatic HER2-low and HER2-ultralow breast cancer patients who have received at least one endocrine therapy. An FDA decision is expected in H1 2025. Results from three additional Phase III trials, DESTINY-Breast09, -11 and -05 are expected in 2025. Destiny-Breast11 and -05 are evaluating Enhertu for HER2+ breast cancer patients in the neodjuvant and adjuvant setting, respectively. The DESTINY-Breast09 study is evaluating Enhertu as first-line therapy for metastatic HER2+ breast cancer and has the potential to replace Roche's Perjeta in this setting.

The anti-PD-L1 antibody Imfinzi experienced significant growth in 2024 following the first approval for gastrointestinal (GI) cancer indications, namely biliary tract cancer (supported by the TOPAZ trial) and liver cancer (supported by the HIMALAYA trial). In 2025 data AstraZeneca expects the first approval for bladder cancer, a genitourinary indication, supported by the NIAGARA Phase III trial. Phase III trials expected to readout in 2025 include PACIFIC-4 for NSCLC, MATTERHORN and EMERALD-1,3 for GI cancer and VOLGA, POTOMAC and NILE for bladder cancer.

Datroway (datopotamab deruxtecan) is an antibody-drug conjugate targeting Trop-2 being jointly developed with Daiichi Sankyo. Datroway was approved in Japan in December 2024 and is under regulatory review by the FDA for late-line, EGFFR mutated NSCLC and for second-line or later unresectable or metastatic HR+/HER2- breast cancer. A submission to EU authorities for NSCLC was withdrawn following feedback from the EMA although the submission for breast cancer remains under review.

The Phase III AVANZAR study is evaluating Datroway + Imfinzi + carboplatin versus Keytruda + platinum based chemotherapy for the first-line treatment of patients with locally advanced or metastatic NSCLC without actionable genomic alterations. AVANZAR is expecting data in H2 2025 and marks several firsts for Datroway including the first Phase III immune-oncology combination trial, the first of five frontline lung cancer trials as well as the first Phase III study to prospectively validate the TROP2-QCS biomarker. Datroway is also being evaluated for first-line, locally recurrent inoperable or metastatic triple-negative breast cancer in TROPION-Breast02, the first Phase III trial in this setting. The trial is enrolling patients who are not candidates for a PD-1/PD-L1 inhibitor and is expecting data in 2025.

The oral selective estrogen receptor degrader (SERD), camizestrant is being investigated in four Phase III trials across both early stage and metastatic HR+/HER2- breast cancer. In the Phase II SERENA-2 trial, camizestrant showed a clear PFS benefit compared to Faslodex, irrespective of ESR1 mutation status. Importantly, camizestrant reported low rates of GI toxicity, meaning patients could remain on treatment for longer. SERENA-6 is the first Phase III trial for camizestrant and is enrolling frontline patients. The trial will readout in 2025 and will mark the first step towards establishing camizestrant as a potential best in class oral SERD and as a new backbone endocrine therapy.

The novel aldosterone synthase inhibitor (ASI) baxdrostat is in Phase III development for hypertension and carries multi-blockbuster potential. In 2025 AstraZeneca expects data from BAX HTN a Phase III trial evaluating baxdostat monotherapy in patients with uncontrolled hypertension on two or more medications including patients with resistant hypertension. Baxdrostat has reported encouraging blood pressure reduction in Phase II studies and benefits from a longer half-life compared to other ASIs which may translate to better 24-hour systolic blood pressure control. A Phase III study of baxdrostat in a fixed-dose combination with Farxiga for patients with kidney disease and high blood pressure was initiated in 2024, one of several combination studies initiated in the cardiovascular, renal and metabolism space reflecting the portfolio strength built in this area.

In the rare disease space, the Phase III CALYPSO trial is evaluating eneboparatide for hyperparathyroidism. Eneboparatide aims to address three clinical priorities for patients, including normalizing serum calcium, decreasing urinary calcium excretion and preserving bone mineral density. Eneboparatide has the potential to be differentiated across all three clinical priorities and has blockbuster potential.

Another asset in the rare disease space is efzimfotase alfa (ALXN1850), a next generation hypophosphatasia medicine developed as a follow-on to Strensiq. Compared to Strensiq, subcutaneous efzimfotase alfa is characterized by lower injection volume, less frequent injections and improved manufacturing process. These innovations have

the potential to broaden the addressable population by as much as six times that of Strensiq making this a blockbuster opportunity. Two Phase III trials, HICKORY (in adolescent and adult patients not previously treated with Strensiq) and CHESTNUT (in pediatric patients previously treated with Strensiq), are expected to readout in 2025.

Looking beyond 2030, AstraZeneca is interested in combining bispecific antibodies such as rilvegostomig (PD-1/TGIT) or volrustomig (PD-1/CTLA-4) with antibody-drug conjugates. This combination is expected to beat PD-1/PD-L1 monoclonal antibodies and then also displace classic chemotherapy if these can be combined with biomarkers for a more precise approach. With multiple high value Phase III readouts expected in 2025, AstraZeneca appears to be on track for its goal of delivering at least 20 new medicines by 2030.

Intuitive Surgical

At the 2025 J.P. Morgan Healthcare Conference, Intuitive Surgical CEO Gary Guthart provided an update on the company's 2024 performance and strategic priorities for 2025. The company reported 2.7 million procedures performed globally, reflecting 17% growth year-on-year, supported by the placement of nearly 1,800 new da Vinci systems, bringing the installed base to 11,000 units. The launch of the da Vinci 5 system was a key focus, featuring advancements such as force feedback and significantly increased computing power aimed at improving surgical performance and supporting future features, including automation and advanced analytics.

Recurring revenue, comprising instruments, accessories, service agreements, and leasing, accounted for 84% of total revenue. Growth in general surgery continued to drive US performance, while international markets such as Japan, Germany, and the UK saw increased adoption, particularly in non-urology procedures. However, the company noted economic pressures in China due to domestic competition and weakened capital budgets in Europe. The Ion platform for lung biopsies saw 78% procedure growth and is expected to contribute further as new indications are approved. For the rest of this year, the company projected 13-16% procedure growth, supported by the broader rollout of da Vinci 5 and continued expansion of the Ion and SP platforms. Intuitive also outlined its strategy to recondition older Xi systems for cost-sensitive markets and outpatient centers.

Additionally, management highlighted continued investments in R&D, manufacturing, and regulatory efforts to scale operations and meet global demand. While acknowledging macroeconomic challenges, Guthart emphasized the company's focus on building long-term value through clinical and operational improvements, expanded system capabilities, and deeper market penetration, with a strong emphasis on surgical data science and AI-driven insights.

Merck & Co.

Merck & Co.'s (MRK) CEO and Chairman Robert Davis, along with Dr. Dean Li, President of Merck Research Laboratories, began their fireside chat at the 2025 JP Morgan Healthcare Conference by reflecting on the company's progress in executing strategic priorities over the past three and a half years, since their joining. According to Davis, Merck & Co. has delivered consistent year-on-year revenue growth, achieving a 7% increase between 2023 and the 2024 midpoint guidance value of $63.9bn. However, this growth rate is slightly lower than the 12% and 9% increases seen in previous years. The company has nearly tripled its number of investigational assets in Phase III development, increasing from nine in 2021 to 26 in 2024, expanding diversity within oncology, as well as other therapeutic areas. To achieve these milestones, Merck & Co. has invested approximately $40 billion in strategic business development over this period, which includes the acquisition of EyeBio in July 2024 for $3 billion. This acquisition adds Restoret to Merck's pipeline, a novel late-phase candidate for diabetic macular edema and neovascular age-related macular degeneration.

Merck & Co.'s robust oncology franchise, anchored by the blockbuster immune checkpoint inhibitor Keytruda, has been a key driver of its sustained success. However, with Keytruda's US patent set to expire in 2028, the company highlighted its proactive steps to manage the upcoming loss of exclusivity. Notable milestones for Keytruda include two strategic approvals from the EC for new indications in gynecologic cancers. The approvals are based on the Phase III KEYNOTE-868 trial for patients with advanced or recurrent endometrial carcinoma and the Phase III KEYNOTE-A18 trial for patients with Stage III-IVA locally advanced cervical cancer. Additionally, Merck & Co. highlighted the promising topline results from the pivotal Phase III MK-3475A-D77 trial, which demonstrates the noninferiority of subcutaneous Keytruda administration in patients with NSCLC. The subcutaneous formulation is expected to receive approval ahead of schedule in 2025 and could enhance Keytruda's uptake across all approved indications due to its more convenient administration compared to intravenous dosing.

Aside from Keytruda, the FDA granted priority review for the HER3-DXd ADC patritumab deruxtecan for patients with locally advanced or metastatic EGFR-mutated NSCLC. Moreover, the company entered into a strategic global development and commercialization agreement with Daiichi Sankyo to investigate MK-6070 in combination with ifinatamab deruxtecan in SCLC. In terms of late-phase pipeline development beyond 2025, Merck & Co. announced the initiation of four pivotal Phase III trials investigating bomedemstat, nemtabrutinib, MK-2870, and MK-5684 across various hematologic malignancies and solid tumors. Given these advancements, Merck & Co. has updated its forecasted oncology opportunity going into the mid-2030s from $20bn to over $25bn.

Outside of oncology, Merck & Co. reported significant progress in its cardiometabolic portfolio. The company secured FDA and EC approval for Winrevair for pulmonary hypertension patients, based on the Phase III STELLAR trial. In December 2024, Merck & Co. signed an exclusive global license agreement with Hansoh Pharma for HS-10535, an investigational oral GLP-1 receptor agonist, strengthening its presence in obesity and metabolic treatments. Furthermore, topline results are expected in 2025 for key pipeline therapies: efinopegdutide, a GLP-1/glucagon receptor dual agonist, for metabolic-associated steatotic liver disease and nonalcoholic steatohepatitis, and enlicitide decanoate for atherosclerotic cardiovascular disease. Merck & Co. also raised its mid-2030s sales outlook for immunology candidates to over $5bn, highlighting advancements in the immunology space. This value creation is based upon the FDA approval of Capvaxive for preventing invasive pneumococcal disease and pneumococcal pneumonia in adults, based on the Phase III STRIDE-3, STRIDE-5, and STRIDE-6 trials. While Gardasil, Merck's HPV vaccine, experienced a 10% year-on-year revenue decline in Q3 2024 due to decreased use in China, the downturn is expected to be offset by the vaccine's label expansion for patients aged 9 to 26 in China in 2025. The company is also progressing with its monoclonal antibody clesrovimab for RSV protection with a PDUFA date set for June 10th 2025, based on positive results from the Phase IIb/III CLEVER trial and ongoing data from the Phase III SMART trial.

In their concluding remarks, Merck & Co.'s leadership expressed confidence in the company's ability to remain a sustainably growing organization well into the next decade and beyond. They emphasized that Merck's evolving portfolio is on track to become 'the most diversified portfolio' in the company's recent history.

Novartis

At the 2025 JPM Healthcare Conference, Novartis' CEO Dr. Vas Narasimhan kicked off the presentation with transformations the company has undergone over the past decade, beginning with huge changes to the Novartis portfolio in 2014, and the spin-offs of Alcon, Sandoz and Haleon (with GSK and Pfizer) to create a company with a 100% focus on innovative medicines by 2023. This slimmed-down portfolio does not appear to have significantly impacted operational performance, with increasing net sales and core operating costs noted in Q3 2024. Dr. Narasimhan also remarked that while transformations have taken place, cash flow over the first three quarters of 2024 surpassed cash flow in 2020 prior to both the spin-offs of Alcon and Sandoz, highlighting continued growth within the company. Looking ahead, Novartis remains committed to the development of its assets and advanced technologies in core therapeutic areas (immunology, cardiovascular-renal-metabolic disease, neuroscience and oncology), in addition to finding the right industry partnerships and delivering high-value medicines in its priority markets in the US, China, Germany and Japan.

Novartis' current portfolio includes 13 in-market brands, eight of which are estimated to be capable of generating over $3 bn in peak sales, with over 30 potential high-value assets in the pipeline across multiple technology platforms. This pipeline has been streamlined through a 40% reduction in the number of clinical stage projects in Novartis' portfolio, increasing resources and capabilities available to higher priority pipeline products by almost 50% in Q3 2024 compared to the same quarter of 2021, which the company CEO remarked allows Novartis to accelerate key projects through strategic investments. Plans are in place to focus on the company's technology platforms across all therapy areas, with a long-term commitment to developing RNA, radioligand, cell and gene therapies to drive growth. This growth will also be driven by de-risked in-market brands such as Kisqali, Scemblix, Pluvicto and Kesimpta, as well as select pipeline assets. Through to 2029, with the anticipated market entry of generics for key brands Entresto, Promacta and Tasigna, Novartis will rely on specific assets to act as growth drivers to maintain or outperform its 5% CAGR projection. Strong new-to-brand prescription (NBRx) shares for Kisqali (50% leader in metastatic breast cancer and 52% in early breast cancer), Cosentyx (60% leader in hidradenitis suppurativa), Pluvicto (35% leader in post-taxane mCRPC) and Scemblix (50% leader in third-line CML) offer confidence that Novartis can deliver on its commercial execution plan while improving its ranking amongst companies in its core geographic markets. Novartis' exciting pipeline includes OAV101 IT, which had a positive readout from the Phase III STEER trial in spinal muscle atrophy having met its primary endpoint, remibrutinib for chronic spontaneous urticaria, pelacarsen for cardiovascular risk reduction, atrasentan for IgA nephropathy and ianalumab for Sjogren's syndrome, and these drugs are expected to contribute to growth beyond 2029 as the lead assets in development.

In addition, at least ten Phase III trial readouts are expected in the next five years for immunology assets, the development of siRNA products for cardiovascular disease will be a focal point for Novartis, aided by the acquisition of Kate Therapeutics, and the company expects to delve into the use of radioligand therapies with over ten programs in the clinic in 2025, including two actinium-based prostate-specific membrane antigen (PSMA) programs, all looking to launch at the end of the decade. Novartis was also a leader over the past two years in mergers and acquisitions with over 30 deals completed to enhance its portfolio, while also remaining a leader in environmental, social and governance ratings, creating a sustainable impact across the healthcare industry.

Dr. Narasimhan concluded the discussion by emphasizing Novartis' continuous investment strategy, growth profile and market leadership which will continue to create value for shareholders in the long term as the company targets big strides over the next five years.

Large Cap

Alnylam Pharmaceuticals

Alnylam Pharmaceuticals is a commercial-stage company with rich pipelines leveraging RNA interference (RNAi) technologies. CEO Yvonne Greenstreet presented an overview, highlighting five approved products, over 25 pipeline developments, and a 33% year-over-year growth in 2024. The company aims for a 40% revenue CAGR through 2025. In 2024, Alnylam achieved several milestones, including positive Phase III data from the HELIOS-B study for treating human transthyretin amyloidosis (ATTR or TTR) with cardiomyopathy (hATTR-CM), an anticipated PDUFA date for vutrisiran on March 23, 2025, and promising results from nucresiran's ANL-TTRsc04 Phase I study and mivelsiran's Phase I study, with the cAPPricorn-1 Phase II study initiated in 2024. Additionally, zilebesiran showed promising blood pressure-lowering effects, and the company expanded four IND applications. Alnylam ended 2024 with $2.7Bn in cash and its four marketed drugs generated a total of $1.6Bn revenues. The TTR franchise achieved a 34% year-over-year growth while the rare disease franchise recorded a 29% increase. Since 2019, the CAGR of the global product sales has been 58%.

The TTR franchise is Alnylam's most revenue-generating portfolio, addressing the rapidly progressive and fatal TTR disease. TTR is caused by misfolded transthyretin protein, impacting the heart, nerves, and gastrointestinal tract. For instance, in the field of hATTR-CM, it is estimated that more than 300,000 people harbor hATTR-CM, with over 80% of patients undiagnosed globally. The dynamics of TTR disease have changed, as the number of diagnosed cases in the US has increased more than tenfold since 2019. Alnylam's Amvuttra is expected to address the underserved ATTR-CM patient population. In the Phase III HELIOS-B study, Amvuttra reduced mortality by 36% in the overall population of patients with ATTR-CM. The experience with ATTR with polyneuropathy (hATTR-PN) demonstrated a high adherence profile for Amvuttra, with 95% adherence. This collective information positions Amvuttra well as a first-line choice for ATTR-CM. Greenstreet plans to capture switch or add-on treatment in stabilized progressors whose disease worsens despite receiving treatments and to drive earlier diagnosis across ATTR-CM patients. The successful launch of Amvuttra in hATTR-CM would be based on its established leadership and experience in the hATTR-PN sector, with an estimated market share of over 80%.

Greenstreet estimated that over 99% of eligible hATTR-PN patients have coverage for Amvuttra, with approximately 70% having zero out-of-pocket costs. The market access of Amvuttra for hATTR-PN smoothens the access for hATTR-CM, as Alnylam has established payer relationships over time. Amvuttra is on track to launch in the US for hATTR-CM with a PDUFA date of March 23, 2025, and in Japan and Germany in the second half of 2025. Alnylam plans to launch a Phase III study (ALN-TTRsc04) for the next-generation pipeline nucresiran in hATTR-CM in the first half of 2025.

Regarding pipeline development, Greenstreet highlighted three pipeline drugs: zilebesiran, mivelsiran, and ALN-HTT02. Zilebesiran has demonstrated promising results for treating hypertension, with a 15 mmHg reduction in blood pressure as a monotherapy from the KARDIA-1 study. Its Phase II KARDIA-3 data on high-risk cardiovascular disease patients with two or more antihypertensives are expected in the second half of 2025, and a Phase III CVOT study is planned to commence in the second half of this year. Mivelsiran addresses the upstream cause of neurodegenerative diseases by targeting and reducing amyloid biosynthesis proteins. The pipeline drug is advancing its cAPPricorn-1 Phase II study in Alzheimer's disease and cerebral amyloid angiopathy (CAA), with an ongoing Phase I study in early-onset autosomal dominant Alzheimer's disease (EOAD) patients. Alnylam is testing ALN-HTT02 in a Phase I study for treating patients with Huntington's disease. Detailed updates on these pipeline drugs will be disclosed on Alnylam's R&D day next month.

The year 2025 is poised to be an inflection point for Alnylam, marked by several significant milestones. These include regulatory approvals and the launch of Amvuttra for hATTR-CM in the US, Japan, and Germany, as well as one product approval in collaboration with Sanofi. Additionally, the company plans to initiate two Phase III trials (ALN-TTRsc04 and CVOT), release the KARDIA-3 Phase II data, file at least four INDs, and achieve non-GAAP profitability following the launch of Amvuttra for hATTR-CM. Alnylam has also upscaled its guidance for 2025, projecting a CAGR of over 40% through the end of the year.

BioMarin Pharmaceuticals

BioMarin's (BMRN) opening remarks centered on celebrating the novelty, profitability, and growing facets of their business base, including traditional enzyme-based therapies and protein analogs designed to treat genetic conditions. From Q3 2023 to Q3 2024, the company experienced double digit revenue growth, with a 19% increase from $1.8bn to $2.1bn. To accelerate this growth, clinical and regulatory milestones for the next 18 months were outlined, including 5 additional indications for Voxzogo and a supplemental filing from Phase III data in adolescents for Palynziq. Voxzogo (vosoritide), an analog of C-type natriuretic peptide (CNP), for achondroplasia has been BioMarin's most successful launch so far. The therapy continues to gain traction, treating approximately 32% of the available population in the major markets, including the US, Europe, Japan, Australia and Canada.

New international treatment guidelines have furthered these efforts by promoting the early initiation of therapy based on additional therapeutic benefits beyond height discovery. Plans were outlined to expand geographic reach, with the remaining markets to be added by 2027, for a total of 24,000 infants and children. As part of the 5

additional indications for Voxzogo, Phase II data was expanded on for the treatment in hypochondroplasia, targeting approximately 14,000 of the most severely impacted patients, with an estimated launch in 2027.

BioMarin has many top-line results anticipated in 2025, including Phase III results for Palynziq in PKU, Talzenna in prostate cancer and Phase I/II results for early-stage asset BMN 351 in Duchenne Muscular Dystrophy (DMD).

The majority of BioMarin's pipeline is in the early stages and represents a diverse array of therapy areas and treatment options, from the next-generation CNP BMN 333 for multiple growth disorders to the oligonucleotide BMN 351 to address DMD. Both assets were discussed alongside their positive proof of concept data, with launches anticipated in 2030 and 2028, respectively, if late-stage development is successful.

By accelerating R&D investment in large, unaddressed patient populations and launching and expanding the base indications of their potential blockbuster, Voxzogo, Biomarin hopes to attain large BioPharma peer profit margins of approximately 40% in 2026 and profits of approximately $4bn in 2027.

Bristol Myers Squibb

A year on from his appointment as BMS' CEO, Chris Boerner kicked-off this year's JPM conference by highlighting his company's efforts to write the next chapter in its history. At last year's JPM, he outlined his company's planned journey to become one of the fastest growing in the field in this decade, with a focus on key growth brands and the delivery of mid-late-stage pipeline assets.

This year, he highlighted the key medicines set to drive BMS' growth portfolio, as well as the company's multiple expected registrational readouts that will define their pipeline's potential and will ensure the focused execution of their goals.

Last year saw BMS reach double-digit sales growth, with two new brands already making their mark following recent approvals — Cobenfy, FDA-approved in September 2024, and Opdivo Qvantig, a subcutaneous formulation of Opdivo FDA-approved in December 2024 for all Opdivo's original indications.

Antipsychotic drug Cobenfy, approved for the treatment of schizophrenia, came to BMS through their $14 billion acquisition of Karuna Therapeutics, and while still new to the market, BMS' billion-dollar bet seems to be paying off, with the drug already gaining traction, and according to Boerner, already ahead of branded schizophrenia launch benchmarks in terms of prescriptions. With its coverage expected to increase in the

second half of 2025 for schizophrenia, BMS' hopes for Cobenfy include several additional indications with multi-billion-dollar peak sales over the decade. Cobenfy is currently in three registrational trials for Alzheimer's disease psychosis and one for adjunctive schizophrenia, for which readouts are expected in the next 24 months.

In addition, there are planned registrational trials for bipolar I disorder, Alzheimer's disease agitation and cognition, and autism spectrum, which will all readout from 2027 to 2029 and have the potential to significantly boost Cobenfy's commercial outlook.

BMS' legacy portfolio — which includes Eliquis, Revlimid, Pomalyst and Sprycel— is expected to be significantly impacted by loss of exclusivity, and in his presentation, Boerner indicated that BMS will capitalize on newer assets to counter the negative impact of generics.

The company's growth portfolio, comprising 14 brands, will rely on five key products — Reblozyl, Camzyos, Breyanzi, Opdualag, and Cobenfy — which are all expected to generate over 50% of the company's revenues in 2025. BMS is also banking on the growing opportunities presented by oral factor XIa inhibitor milvexian as anticoagulant for thrombotic diseases, and LPA1 antagonist admilparant for pulmonary fibrosis, both of which are aimed at addressing significant unmet needs in large, and thus lucrative, indications.

With expected cost savings of $1.5 billion by the end of 2025, BMS plans to reinvest in high return growth initiatives, prioritizing the highest value programs. The company's focus is shifting towards a younger, more diversified and de-risked portfolio, which is balanced across leading therapy areas, and their ambitious plans to secure 30 new label expansions over the next five years have the potential to offset the significant losses the company expects from the genericization of its legacy portfolio.

As BMS continues to navigate a rapidly evolving pharmaceutical landscape, Boerner's vision underscores a strategic pivot towards innovation and sustainability. By leveraging a combination of pipeline expansion, strategic acquisitions, and a disciplined focus on execution, the company is positioning itself for long-term success. While the challenges of patent expirations loom large, BMS' ability to capitalize on high-growth assets and emerging therapies will be critical in defining its trajectory in the years ahead.

If the company's execution aligns with its vision, BMS could well establish itself as one of the dominant players in the biopharma industry for the foreseeable future.

Cooper Companies

At the J.P. Morgan Healthcare Conference on January 14, 2025, CooperCompanies outlined its successes and outlooks for its two CooperVision and CooperSurgical business divisions. Over the past ten years, CooperCompanies have successfully more than doubled its revenue from $1.8 billion in 2015 to $3.9 billion in 2024. The company anticipates further growth in 2025, with an estimated revenue guidance of $4.1 billion.

The company emphasized its long term growth strategy to execute on strategic goals and drive shareholder value during the presentation. This strategy includes investing in and driving organic growth and gaining market share through an expansion of their manufacturing capabilities, optimization of technology investments, development and launch of new products, and extensive employee training.

The company's vision division, CooperVision, has had a long history of success in both sales and market share growth. CooperVision is currently the #1 contact lens company in the world with 34% of all lens wearers using CooperVision lenses. CooperVision is responsible for the development of the MiSight 1 day soft contact lenses which is the first and only FDA approved contact lens for myopia control.

With a comprehensive portfolio of treatment option, CooperVision is a global market leader in myopia management. The business has a large global footprint and strong relationships with independent practitioners, distributors and retailers.

CooperCompanies' second business division, CooperSurgical, brought in $1.29 billion in revenue in 2024. CooperSurgical is committed to supporting patients and clinics by driving innovation, improving access, and addressing the challenges of declining birth rates.

The company holds over 600 products in more than 130 countries, and has completed over 40 acquisitions since 1990. It is currently the #1 fertility company in the medical device industry, boasting the broadest portfolio that includes consumables, capital equipment, reproductive genetic testing, egg and sperm donation, and cryopreservation. In the future, the company is looking to launch new products and services, open new donor sites, and expand geographically.

CooperSurgical has also entered into a collaboration with the American Society for Reproductive Medicine and the Society for Reproductive Biologist and Technologists or a newly formed Clinical Embryology Learning Lab.

Genmab

In his JPM 2025 opening remarks, Genmab's CEO Jan van den Winkel reiterated his company's focus to evolve into a "fully integrated biotech innovation powerhouse", focussing on improving the lives of patients through "innovative and differentiated antibody therapeutics". The company's vision is that by 2030, their KYSO ("Knock Your Socks Off") antibody medicines will fundamentally transform the lives of patients with cancer and other serious diseases.

In addition to eight approved drugs, which are either fully-owned or are owned by third parties but were created by Genmab or incorporate Genmab's innovation — Tivdak, Darzalex/Darzalex Faspro, Rybrevant, Kesimpta, Tepezza, Tecvayli, Epkinly, and Talvey — the company also boasts an innovative clinical pipeline, with agents in Phase I, II and III trials, as well as early clinical development. Aside from the brands, which continue to be assessed for line/label expansions, there are twelve drugs owned by Genmab at ≥50%, and three royalty medicines which are owned by partners, but which will generate royalty revenue in case of approval.

Consensus forecasts for Genmab's royalty medicines, namely Darzalex, Kesimpta, Tepezza, Tecvayli, Talvey and Rybrevant, project significant revenues of nearly $18 billion in 2024, and the royalties that will flow to Genmab are expected to be significant (from mid-single digit to double-digit percentage in royalties), which will help the company drive its development further and transform pipeline therapies into revenue- generating ones.

Co-developed with AbbVie, bispecific antibody Epkinly/Tepkinly, is now approved in the US, Europe and Japan for relapsed/refractory (R/R) DLBCL, as well as for R/R FL in the US and Europe, and with a subcutaneous delivery that distinguishes it from its bispecific competitors, is considered a great addition to the treatment armamentarium.

Following the drug's accelerated approval in the US, Genmab is now pushing Epkinly's development plan further, with five Phase III trials across two indications (DLBCL and FL), in both the front-line setting (which, with a larger target population, is more lucrative than the third-line DLBCL and FL, the drug's current label) and the R/R setting.

Genmab is also expanding the drug's early phase development to other indications beyond DLBCL and FL, with CLL and B-cell NHL included in their trials.

Genmab's collaboration with AbbVie seems to be successful for Epkinly, as noted in the presentation, over 90% of the drug's global revenues are from the US and Japan, and the company expects revenues to increase once a Japanese approval is granted for use in R/RFL. Should Epkinly be approved for DLBCL and FL in earlier lines of treatment, Genmab highlights a $3 billion opportunity for Epkinly by the end of 2031.

Tivdak, co-developed and co-promoted with Pfizer, is the first and only antibody-drug conjugate FDA-approved under the accelerated program for second-line recurrent or metastatic cervical cancer with disease progression on or after chemotherapy. In a bid to expand Tidvak's commercial outlook, Genmab is pursuing a broadening of its label to include early lines of cervical cancer, as well as other solid tumors. Genmab also expects a Japanese regulatory approval in the first half of 2025 for use in cervical cancer patients. Since its launch in 2021, Tivdak has seen 12 consecutive quarters of growth, with $91 million in revenues in the first nine months of 2024.

Through their acquisition of ProfoundBio in 2024, Genmab capitalized on FRα-targeting TOPO1 antibody-drug conjugate rinatabart sesutecan (Rina-S), which is now wholly owned by Genmab. The drug is in Phase II and III development for FRα-expressing tumors, with an ongoing Phase III trial in second-line platinum-resistant ovarian cancer in all comers, regardless of FRα expression. With planned launches in platinum-resistant and platinum-sensitive ovarian cancer, as well as endometrial cancer, Genmab highlights a $2 billion opportunity for revenue from Rina-S by the end of 2031.

Ending a collaboration with BioNTech in 2024, Biogen assumed full ownership of bispecific checkpoint inhibitor acasunlimab (DuoBody-PD-L1x4-1BB,) currently in Phase III development in second-line and beyond NSCLC patients who have progressed on a checkpoint inhibitor. Encouraging early-phase data from NSCLC presented at ASCO 2024 highlight acasunlimab's potential, with a commercial opportunity at launch of over $1 billion.

In his presentation, van der Winkel indicated that additional trials for acasunlimab will be announced in due course. In closing, van den Winkel indicated that the company's operating profit for 2024 is expected to increase by 17% from its 2023 value, to approximately DKK 6.3 billion ($0.87 billion).

GSK

GSK's CEO Emma Walmsley opened the presentation by highlighting GSK's robust performance, reflecting its strategic emphasis on specialty medicines and vaccines. GSK's operational efficiency led to twice-upgraded guidance for 2024, projecting annual sales growth between 7-9% and profit growth of 11-13%. In 2024, the company achieved a 9% growth in sales and a remarkable 19% growth in core operating profit over the first nine months. This strong growth was driven by specialty medicines, which grew more than 20% in the first nine months of 2024, with the HIV franchise growing by more than 13% and the respiratory/immunology franchise increasing by more than 15%. Oncology sales doubled, reaching £1Bn.

Walmsley provided an ambitious outlook for GSK's 2025 plan, including launching five products. Blenrep, an off-the-shelf antibody-drug conjugate (ADC) for treating multiple myeloma, is anticipated to bring more than £3Bn in peak years due to its significant reduction in the risk of death by 42% and increased overall survival by 33 months as a second-line therapy compared to the standard of care. The drug is in the middle of a Phase III study (DREAMM-10) as a first-line therapy. Depemokimab is a long-acting anti-IL-5 antibody designed for once every-six-month dosing. The drug showed a 72% reduction in asthma exacerbations requiring hospitalizations. Meanwhile, depemokimab is being tested in a Phase III trial for treating patients with chronic obstructive pulmonary disease (COPD). The development of depemokimab was to extend the IL-5 franchise led by Nucala. Nucala, an anti-IL-5 antibody approved for treating eosinophilic asthma, is anticipated to stride into COPD treatment in 2025. The combined peak sales from depemokimab and Nucala are projected to be more than £4Bn in peak years. The other two anticipated launches in 2025 are gepotidacin for treating uncomplicated urinary tract infection and MenABCWY, a 5-in-1 vaccine against meningitis.

In 2024, GSK disclosed 13 positive Phase III readouts and plans to develop additional new assets, including two ADCs (B7H3 and B7H4). The B7H3-targeted ADC received a breakthrough therapy designation from the FDA for osteosarcoma, following the same designation for treating small cell lung cancer. Its pivotal trials will commence in 2025 and 2026. Walmsley mentioned that B7H4 ADC has the best-in-class potential for treating ovarian and endometrial cancers, with additional opportunities in treating other solid tumors. The first launch of these ADCs is anticipated in 2027. In respiratory assets, camlixipant's Phase III readout will be disclosed at the end of 2025 for treating chronic cough. The proof-of-concept data of oligonucleotide GSK4532990 on patients with

metabolic dysfunction-associated steatohepatitis (MASH) and alcohol-related liver disease (ALD) will be revealed in 2026 and 2027, respectively. GSK aims to launch its HIV pre-exposure prophylaxis (PrEP) therapeutic in 2026 with a once-every-four-month (Q4M) formula. The registrational study for the Q4M treatment will start in the second half of 2025, with a Q6M treatment regimen planned in 2026.

In 2024, GSK bolstered its platform through targeted acquisitions, including assets from Hansoh, IDRx, and Chimagen Biosciences, enhancing its oncology and immunology portfolios. GSK secured full rights to mRNA candidate vaccines from CureVac and acquired Elsie Biotechnologies for the design and development of oligonucleotides to strengthen its platform capabilities. Collaborations with Flagship Pioneering provided access to over 40 bioplatform companies, broadening its innovation scope. Walmsley was confident in delivering growth and returns to shareholders as well as short, medium, and long-term commitments in the years to come.

The executive team provided insights into the new acquisition of IDRx, which is developing IDRX-42 for gastrointestinal stromal tumors (GIST). Approximately 80% of GIST patients have KIT gene mutations, with 90% experiencing disease progression despite standard treatments. In the U.S., the addressable patient population is 6,000. Current therapies, such as imatinib, target KIT exon 9 and 11 mutations but fail to address resistance mutations in exons 13 to 17, leading to disease progression within 19 months. Additionally, 40% of imatinib-treated patients experience Grade 3 toxicity, while Sutent has a 60% incidence. IDRX-42 effectively targets all relevant KIT mutations with high kinase selectivity and a favorable toxicity profile, enabling prolonged dosing. Strategically, the acquisition aligns with GSK's portfolio, as healthcare providers likely to adopt IDRX-42 overlap with those prescribing Jemperli, GSK's colorectal cancer therapy. The ongoing study includes patients previously treated with multiple therapies, offering valuable insights into its positioning in GIST treatment.

Incyte

Incyte CEO Hervé Hoppenot presented a talk entitled "2025: A year of defining catalysts". Hoppenot started by noting that the total revenue for the first 9 months of 2024 was $3.1bn, an increase of 14% year over year. While revenue from Jakafi (ruxolitinib tablets) grew 6% year over year, revenue from Opzelura (ruxolitinib cream) grew by 52% year over year reflecting diversification of revenue and international expansion. On the regulatory side, Niktimvo, a monoclonal antibody targeting CSF-1R developed with partner Syndax, was approved for 3L+ chronic graft-versus-host disease. There were also three regulatory submissions: a sNDA was submitted for Opzelura for pediatric atopic dermatitis, a sBLA was submitted for Zynyz, an PD-1 antibody for squamous cell anal carcinoma and for Monjuvi, an anti-CD19 antibody for relapsed/refractory follicular lymphoma. These submissions are expected to lead to three

launches in 2025 which, with the launch of Niktimvo, will help grow revenue. While all four launches are expected to have modest sales potential, together they are expected to reach ~$1bn in revenue by 2029.

Four pivotal trial readouts are expected in 2025. Povorcitinib is an oral JAK1 inhibitor and is part of Incyte's effort to further expand its portfolio into the dermatology market. A Phase III trial is evaluating povorcitinib for moderate to severe hidradenitis suppurativa (HS) and is expecting a readout in 2025. Other expected pivotal trial readouts are Opzelura for prurigo nodularis, Monjuvi for 1L DLBCL and a trial comparing the bioequivalence of once daily ruxolitinib (ruxolitinib XR) to Jakafi (administered twice daily).

For the rest of the presentation, Hoppenot focused on povorcitinib which is expected to be a large market opportunity, an mCALR antibody which could transform treatment of myelofibrosis and essential thrombocytopenia (ET) and a CDK2 inhibitor (CDK2i) which is expected to be first-in-class and has a large potential in ovarian cancer.

Povorcitinib is differentiated by its selectivity for JAK1 over JAK2 and is expected to have less side effects than other JAK inhibitors. In addition to the Phase III trial for HS, povorcitinib is being evaluated in Phase III trials for vitiligo and prurigo nodularis with data expected in 2026. For all three indications povorcitinib is being positioned as the first oral therapy. For HS, povorcitinib will be competing with injectable monoclonal antibodies and placebo-controlled Phase II data has shown biologic-like efficacy but with a faster onset for relief of pain. While Opzelura is approved for vitiligo, povorcitinib is being evaluated in patients with more extensive vitiligo that covers a body surface area ≥5% and so cannot be treated with a topical. Approval of povorcitinib for vitiligo and prurigo nodularis is expected in the 2027-2028 time frame.

mCALR is a monoclonal antibody targeting mutant calreticulin, a driver mutation for myeloproliferative neoplasms such as myelofibrosis and ET where the mutation is present in 35% and 25% of patients, respectively. Phase I proof of concept (POC) data are expected in 2025 and if mCALR can kill enough mCALR expressing cells to significantly reduce the allelic burden of mCALR, it has the potential to be a functional cure for these diseases. Hoppenot noted that while Jakafi reduces disease symptoms, it does not eliminate the disease clone and is not curative. Approval of mCALR is expected in the 2027-2028 time frame.

Finally, CDK2i (INCB123667) is targeting Cyclin E overexpressing tumor types. At ESMO 2024, encouraging Phase II data were presented for platinum-resistant, Cyclin E1 overexpressing ovarian cancer (Cyclin E1+ by IHC). Two potentially pivotal studies in the 2L-4L setting are being initiated in early 2025. The first study is a single arm Phase II study with ORR as the primary endpoint and data expected in H2 2026. This trial has the

potential to support a submission for accelerated approval. The second trial is a randomized Phase III trial with chemotherapy as the comparator and PFS as the primary endpoint. Data for the Phase III trial are expected in H2 2027. Finally, a randomized Phase III trial is expected to initiate in 2025 in the 1L homologous recombination deficiency negative setting. The trial will evaluate CDK2i + bevacizumab versus bevacizumab alone as maintenance after 1L chemotherapy. The primary endpoint will be PFS and data are expected in 2029.

Hoppenot concluded by noting that with four product launches expected in 2025 and five product launches expected in 2026 (CDK2i for ovarian, Opzelura for prurigo nodularis, povorcitinib for HS, ruxolitinib XR for Jakafi indications and Monjuvi for 1L DLBCL), Incyte is well positioned for growth in 2025 and beyond.

Neurocrine Biosciences

Neurocrine has discovered and developed four first-in-class FDA-approved neurology assets. To start this presentation, Chief Executive Officer Kyle Gano focused heavily on two products: Ingrezza, which leads Neurocrine's portfolio as the first medication approved for tardive dyskinesia (TD), and Crenessity, which was recently launched in the US for classic congenital adrenal hyperplasia (CAH). These drugs have given Neurocrine a strong platform for investment. Sales of Ingrezza reached $613 million in Q3 2024, representing a 26% year-over-year growth, with Neurocrine boasting a 2024 net product sales guidance of $2.30–$2.32. Considering 80% of the estimated 800,000 patients with TD in the US are not yet receiving a VMAT2 inhibitor, there remains considerable commercial opportunity until Ingrezza loses market exclusivity in 2038.

Crenessity received approval on December 13, 2024, nearly two weeks prior to its PDUFA date. It is the first medication approved as an adjunct treatment to glucocorticoid replacement to control androgens in adult and pediatric patients ages 4+ with classic CAH, and the CEO spoke very favorably of the clean, broad FDA label. Crenessity provides a more targeted approach to CAH treatment, which mitigates the long-term risks associated with glucocorticoid therapy. In the Q&A session, the speaker described a favorable response to the drug so far, although the launch is still at a very early stage. Payers were previewed with the launch plan for Crenessity last year, but engagement is continuing following approval. Crenessity is not yet listed on any formularies. It is expected that the vast majority of patients will pay $12 or less per month for the drug.

Neurocrine currently has 12 pipeline programs ranging from Phase I to III, which includes an industry-leading portfolio of muscarinic compounds selective for the M1 or M4 receptors, with one asset possessing dual M1/M4 selectivity. Positive Phase II data were recently released for the company's leading muscarinic agonist, NBI-‵568, which is a highly selective orthosteric M4 agonist for schizophrenia. The drug is differentiated

from competitors as it does not require the use of acetylcholine. Neurocrine plans to deliver a new medicine to the market every two years by investing 30% of its revenue back into R&D. Part of the plan is to move away from a reliance on external innovation towards an internal discovery engine. This will include a shift from small molecules towards multimodality molecules, from unvalidated to validated targets, and from symptomatic to disease modifying therapies. Per year, the company aims for four new Phase I initiations, two new Phase II initiations and three new Phase III programs.

As it stands, Neurocrine has completed end of Phase II meetings with the FDA for osavampator in major depressive disorder (MDD) and NBI-'568 in schizophrenia, with registrational Phase III programs expected to start in the first half of 2025. Phase II studies evaluating NBI-'568 in bipolar mania and NBI-'570 in schizophrenia are expected to start in the second half of 2025. Phase III data for valbenazine as an adjunctive treatment in schizophrenia and dyskinetic cerebral palsy are expected in 2025, as well as Phase II data for NBI-'770 in MDD. Additionally, four muscarinic drugs have entered into Phase I development. New programs advancing from preclinical to clinical development in 2025 include NBI-'1435, a peptide for CAH, and gene therapies NBIB-'223 (for Friedreich's ataxia) and NBIB-'233 (for Parkinson's disease and Gaucher disease).

Pfizer

At the JPM Healthcare Conference 2025, Pfizer, led by Albert Bourla, Chairman and Chief Executive Officer (CEO), reaffirmed their belief on achieving the revenues ranging between $61-$64 bn in 2025. Besides the COVID-19 sales (Paxlovid) offset, the company will also be facing a decline of about $18bn owing to the Inflation Reduction Act (IRA) in this year. But they are confident of combating it with the sales from Seagen's antibody-drug conjugate (ADC) therapies and in-house as well as out-licensed pipeline assets.

Following Mikael Dolsten's departure, Chris Boshoff has been appointed as the new head of R&D head to guide the company towards a more focused and efficient R&D strategy, aimed at delivering profitable new drugs. The company intends to concentrate on advancing its selective pipeline assets that exhibit significant sales potential and Mr. Bourla expressed considerable enthusiasm regarding CDK inhibitors, anti PD-1/PD-L1 therapies, and vaccine targeting Clostridioides difficile (C. difficile) among others.

The CEO highlighted their assumptions on the market sales of their key products, and shared that the drugs would continue to have market share and sales expansion in 2025. He also reflected upon the $43bn acquisition of Seagen targeted at making oncology a significant growth driver for Pfizer. Seagen's antibody-drug conjugate (ADC) therapie achieved sales of $2.3 bn during the first nine months of 2024 aligning with the

expected goal of contributing more than $10 bn in risk-adjusted revenues by 2030. The company also expects regulatory decisions for Adcetris, Braftovi and Talzenna + Xtandi in diffuse large B-cell lymphoma, BRAF-mutant metastatic colorectal cancer (CRC) and metastatic castration-resistant prostate cancer respectively. Mr. Bourla concluded the presentation by discussing Pfizer's strategic execution of expansion of operating margin to drive shareholder value through 2030.

Sandoz

Chief Executive Officer Richard Saynor began the Sandoz (SDZ) presentation by focusing on the company's position as a top 3 player in the global USD 200 billion generics and biosimilars market. Since its spin-off from parent company Novartis in October 2023, Sandoz successfully launched the number one biosimilar in the US, Hyrimoz® (adalimumab), in the US and Tyruko® (natalizumab) in Europe. Further biosimilar launches are planned for 2025: Pyzchiva® (ustekinumab) (Q1) and Tyruko® (2025 pending FDA approval) in the US, Wyost®/Jubbonti® (denosumab) in the US (Q2 2025) and Europe, and Afqlir® (aflibercept) in Europe. Already leading the biosimilars market in Europe where they operate in over 40 markets, the additional launches may well elevate Sandoz to the top spot in the US as well.

Sandoz momentum in the generics and biosimilars space continues to grow with 28 molecules in its biosimilars pipeline as well as a strong, diverse pipeline of over 450 generics products. GLP-1s present a key opportunity in the generics space (projected to reach over USD 150 billion), and Sandoz intends to maximize its share through internal capabilities and strategic partnerships. Saynor discussed a three-pronged strategy of focusing on the right products, continuous and competitive supply, and the right commercial models.

With a robust pipeline, a strong balance sheet and the resources to attract strong partners, Sandoz is poised to strengthen its position as a top player in the growing generics and biosimilars market which is projected to grow at an annual compound rate of 7% for the next 10 years.

Sanofi

Paul Hudson, Sanofi's CEO, began the company's short presentation today by expressing their excitement for the coming year within the company. Hudson emphasized how hard the company has worked over the last 5 years and how proud they are of the progress that has been made during that time. Sanofi has been strategizing to become a focused science-driven biopharma delivering innovative medicines and vaccines to patients and they believe they will be the world's leading immunology company by the end of the decade.

Hudson gave a brief overview of the success of the business in 2024, including an 11% growth in sales, 3.9 billion dollars of sales of nine newly launched medicines and vaccines, and one new blockbuster drug (Beyfortus). Sanofi expects a strong rebound to continue through 2025 with financial and pipeline progress. Hudson also gave a brief overview of the current standing of the company's pipeline and what investors can expect to see from the company through 2025 and into 2026 which includes multiple expected launches. Although Hudson's coverage of the 2025 pipeline outlook was brief, he stated that a full pipeline update will be given during their next earnings results presentation.

Hudson closed out Sanofi's presentation with a brief overview of what the company accomplished in 2024 and solidified their expectations for the coming year.

Sarepta Therapeutics

At the 2025 J.P. Morgan Healthcare Conference, Sarepta Therapeutics provided a comprehensive overview of their recent achievements and strategic goals. The company reported total net product revenue of $1.79 billion for 2024, with $638 million generated in the fourth quarter alone. ELEVIDYS, their gene therapy for Duchenne muscular dystrophy (DMD), was highlighted as a major growth driver. The company expects U.S. revenue from the therapy to reach $3.1 billion within the first 30 months of its launch, positioning it as one of the strongest gene therapies launches to date.

Sarepta's guidance for 2025 reflects a projected 67% revenue increase, supported by the continued expansion of ELEVIDYS and sustained growth in their RNA-based PMO platform. The company also reiterated its longer-term aspirations, aiming to bring 10 approved therapies to market and achieve $30 billion in annual revenue by 2030.

The presentation also outlined key advancements across their research and development programs. Sarepta plans to initiate phase 1 trials for SRP-9005 (LGMD2C/R5) and their siRNA-based programs in collaboration with Arrowhead, including ARO-DUX4 for facioscapulohumeral muscular dystrophy (FSHD) and ARO-DM1 for myotonic dystrophy type 1. In addition, significant data readouts are expected from the EMERGENE study for LGMD2E/R4 and the EMBARK trial for ELEVIDYS in 2025, alongside updates from studies evaluating the therapy in broader patient populations.

Sarepta also emphasised their continued focus on expanding their manufacturing capacity to meet increasing demand and improve operational efficiency.

Swedish Orphan Biovitrum

Swedish Orphan Biovitrum (Sobi) is a global biopharmaceutical company developing therapeutics for a variety of rare disease such as haemophilia A and B, immune thrombocytopenia (ITP) and paroxysmal nocturnal haemoglobinuria (PNH). Sobi's Chief Executive Officer Guido Oelkers led the presentation and began by describing the accomplishments of the company that occurred during 2024 before moving on to outline the catalysts that are expected throughout 2025.

Sobi is confident in the best-in-class potential of the company's PNH drug Aspaveli/Empaveli (pegcetacoplan) which presented pivotal data from the Phase III VALIANT trial during 2024. Regulatory submissions in Europe and Japan are now expected during 2025 for use in the treatment of C3 glomerulopathy (C3G) and primary immune complex membranoproliferative glomerulonephritis (IC-MPGN), which are both rare kidney diseases with no currently approved treatments. Meanwhile, FDA approvals are expected in the US for Gamifant (emapalumab), for use in primary hemophagocytic lymphohistiocytosis (HLH), and SEL-212, for use in for chronic refractory gout. In 2025, a key data readout is expected for Zynlonta (loncastuximab tesirine) from the ongoing Phase III LOTUS-5 trial in relapsed or refractory diffuse large B-cell lymphoma (DLBCL).

The company was also pleased to have conducted a successful launch in 2024 of its hemophilia A drug Altuvoct (efanesoctocog alfa) which gained approval in Europe in June 2024. It was commercially launched in Germany in July 2024 where it experience rapid uptake and accounted for over 6% of the German hemophilia A market within the first 2.5 months of launch. However, Oelkers did express some disappointment that Vonjo (pacritinib) uptake has been somewhat slower in the US market, since its 2022 approval, but the company has a plan for approaching this. It will focus on building awareness around product in the medical community, increasing education about the high unmet need in myelofibrosis treatment through increased engagement with healthcare professionals, while also broadening the evidence for Vonjo use in myelofibrosis.

Sobi has also seen an impressive 22% growth in total revenues through the first nine months of 2024 compared to 2023, and estimated that full 2024 year revenues for the company are likely to be around SEK 26 billion, which would represent an approximate 19% growth CER.

Teva Pharmaceutical

At the 43rd J.P. Morgan conference, Teva's president and CEO, Richard Francis, looks ahead with optimism on the company's growth in the past couple of years. In only his second year as CEO of Teva, the company has showcased seven quarters of consecutive

revenue growth with a strong portfolio of marketed and promising candidates in the pipeline. The company is executing its Pivot to Growth strategy, focusing on expanding its innovative medicines and biosimilar portfolio while maintaining a strong presence in the generics market. The presentation highlighted Teva's progress toward its 2027 targets, recent successes, and the steps it takes to sustain long-term growth.

Teva's financial performance is on an upward trajectory. The generics business remains a key driver, delivering over $9 billion in revenue for the 12 months ending in September 2024. The company expects free cash flow (FCF) between $1.7 billion and $2.0 billion for 2024, supported by mid-single-digit revenue growth from 2023 to 2027. Teva is making notable strides in margin progression, with the operating margin projected to grow from 27.7% in 2022 to 30% by 2027. The company has also prioritized debt reduction to reduce leverage to 2.0x net debt to EBITDA by 2027 and attain an investment-grade credit rating.

Teva's Pivot to Growth strategy revolves around accelerating its innovative medicines and biosimilar portfolio. Key drugs, such as Austedo, Ajovy, and Uzedy, are experiencing significant revenue growth, and the company is on track to meet its 2024 projections for these products. Originally, peak sales expected from Austedo were restricted to less than
$1.5 billion. However, the drug's 2024 revenue shows an initial outlook of over $1.6 billion, which is 31% higher growth over 2023 and is on track to achieve over $2.5 billion by 2027. Uzedy is also on track to generate around $100 million in 2024, while Ajovy is expected to show a 15% growth compared to 2023, with a $500 million revenue generation expected in 2024.

Stepping up on innovation, Francis was particularly excited about duvakitug in ulcerative colitis and Crohn's disease, which produced positive results in Phase II trials for both indications. Duvakitug, an anti-TL1A, is an exciting prospect in treating fibrotic conditions, and KOLs have shown interest in the mechanism of action. The drug showed potential best-in-class efficacy and was the first placebo-controlled trial of the mechanism in Crohn's disease. Teva and Sanofi are co-developing the drug and target the initiation of Phase III trials in 2025. Another pipeline agent in Teva's arsenal was olanzapine LAI which met its primary endpoint in the Phase III SOLARIS trial with no patients having post-injection delirium/sedation syndrome (PDSS). With a significant market size for the drug, Teva is excited to file the drug with regulatory bodies in 2025, with a potential launch expected in 2026. Although initially put on the back burner, TEV-‘248 (DARI (ICS/SABA)) is expected to be a unique asset with high potential for success. The dual-action rescue inhaler (DARI) for asthma is advancing well in Phase III trials and is positioned as a unique treatment that addresses pediatric and adult populations. The ICS/SABA class is expected to capture 30% of the U.S. asthma market, with DARI playing a key role in this growth.

Teva has a growing pipeline of 18 biosimilar assets across multiple therapeutic areas, with an estimated originator value of $60 billion. Key products include biosimilar denosumab (Prolia), biosimilar aflibercept (Eylea), and biosimilar vedolizumab (Entyvio), developed through collaborations with companies like Alvotech and Samsung Bioepis. Teva's biosimilar business will see several major launches in 2024 and 2025, contributing to the company's long-term revenue growth. The company also focuses on biosimilar market expansion in the U.S. and Europe. The generics business also remains a key cash generator for Teva and the company continues to optimize this business to improve profitability while reducing costs through its global productivity program. Teva's generics business has seen revenue growth of +4% in Q3 2024 compared to Q3 2023 and is expected to return to sustained growth in the coming years.

Teva has taken decisive steps to streamline its portfolio and focus on high-return opportunities. The company is on track to divest its API business and Japan BV unit by the first half of 2025. These divestitures are part of a broader effort to focus on core growth engines and improve profitability. Teva has also been enhancing its launch and supply performance, significantly improving metrics in 2024. This positions the company for greater efficiency in delivering its innovative medicines to the market.

Looking forward, Teva is well-positioned to achieve its 2027 financial targets, which include mid-single-digit revenue growth, 30% operating margin, and 2.0x net debt/EBITDA leverage. Francis emphasized continuing to drive momentum by pushing the marketed drugs while simultaneously developing pipeline assets. He also mentioned that through strategic focus, disciplined execution, and continued innovation, Teva is on track to strengthen its leadership position in the global pharmaceutical industry.

Vertex Pharmaceuticals

Vertex Pharmaceutical’s Chief Executive Officer and President Reshma Kewalramani lead the presentation on the company’s key research and development focuses and the upcoming drug approvals and pipeline advancements that are expected during 2025.

Vertex’s cystic fibrosis drug Alyftrek is a triple-combination therapy containing vanzacaftor, tezacaftor and deutivacaftor which is a once-daily CFTR modulator that has the potential to set a new standard of care in cystic fibrosis treatment. It gained FDA approval in December 2024 and further approvals are expected in 2025 in the markets of UK, EU, Australia, New Zealand, Canada and Switzerland. Another key development focus for the company is in the area of acute pain. Currently in the US, approximately 40 million acute pain patients annually receive a prescription for opioids, which come with a high risk for addiction. The company’s pain candidate suzetrigine is currently filed for

approval in the US for use in moderate-to-severe acute pain, with a PDUFA date set for January 30, 2025. Suzetrigine is a non-opioid pain treatment, which works via pathways in the peripheral nervous system, lessening the risk for addiction. Suzetrigine will be further developed throughout 2025 with ongoing and planned Phase III trials in peripheral neuropathic pain (PNP) conditions such as diabetic peripheral neuropathy (DPN) and lumbosacral radiculopathy (LSR).
The company also plans to expand on its renal franchise during 2025, with ongoing clinical trials for inaxaplin, povetacicept and VX-407 in APOL1 mediated kidney disease (AMKD), B cell mediated renal diseases and autosomal dominant polycystic kidney disease, respectively. There are also plans to expand on the manufacturing capacity for Casgevy, which is approved worldwide for use in sickle cell disease and beta thalassaemia, to support global demand.

Viatris

Viatris, a global healthcare company, has positioned itself as a key player in the pharmaceutical industry by delivering essential medicines and healthcare products to over a billion people annually. The CEO, Scott A. Smith, has been with the company for just over two years and, while introducing the company, gave high praise to his dedicated employees. Scott gave a very brief overview of the company and its goals and achievements in a very short session, with the bulk of the content coming from the Q&A section following the presentation. As the company moves towards 2025 and beyond, it has emphasized a strategy focused on balanced capital allocation and business development, alongside maintaining strong financial performance.

In 2024, Viatris returned to base business growth, increased new product revenue to approximately $600 million, completed divestitures, simplified and streamlined the company while returning over $1 billion to shareholders by share buybacks and dividends, and expanded the company's innovative portfolio. Viatris is already benefiting from its diverse product portfolio and global reach, as evidenced by its financial performance over the last 12 months. The company reported $15.0 billion in total revenues, indicating its strong market position. Additionally, Viatris generated $4.8 billion in adjusted EBITDA, showcasing its profitability and efficient cost management.

The company's free cash flow for the same period stood at $2.4 billion, excluding transaction costs, demonstrating a healthy liquidity position that allows Viatris to pursue further growth opportunities while continuing to deliver returns to shareholders.

The company has a strong generics department with a portfolio of globally iconic brands contributing significantly to its revenue. Geographically, Viatris enjoys a well-diversified revenue mix, which minimizes regional risks and capitalizes on the unique opportunities

that various markets present. This diverse presence in over 165 countries and territories with 26 manufacturing facilities allows Viatris to leverage growth from developed and emerging markets, positioning it to handle market fluctuations more effectively.

Viatris has developed a Balanced Capital Allocation Framework to ensure sustainable growth and profitability. On the Capital Return side, Viatris plans to allocate more resources towards returning capital to shareholders, particularly focusing on 2025. Scott emphasized that a significant aspect of this strategy is the prioritization of share buybacks at the company's current valuation, a move that reflects management's confidence in its future performance. Additionally, Viatris remains committed to delivering quarterly dividends and ensuring consistent returns to investors. Regarding Business Development, the company's growth strategy revolves around acquiring innovative, patent-protected assets that are either commercially available or near-market launch. These acquisitions are expected to contribute revenue between 2025 and 2027, a key growth period for Viatris.

By the end of 2024, the company will have paid down over $10 billion in debt, which Scott believes will allow Viatris to continue building and supporting the base business, building and strengthening the pipeline and returning capital to shareholders. Regarding headwinds, Scott spoke about the FDA import alert set on a manufacturing facility in Indore, India, which the FDA flagged after inspection. He highlighted that Viatris is remediating the issues with the site and is in active discussions with the FDA. This has affected 11 products in the US. However, four products are on the exempt list, with the company expecting to add more.

Doretta Mistras, the company's Chief Financial Officer, mentioned that the company expects strong performance stemming from Europe and China. Major contributions are also expected from the generics portfolio and new expected launches that could generate around $450-$550 million in product revenue.

Viatris's 2025 strategy reflects a clear commitment to balanced capital allocation, continued innovation, and geographic diversification. By prioritizing capital returns through share buybacks and dividends while actively pursuing business development through patent-protected assets, Viatris is well-positioned for future growth. The company's diverse revenue mix and global reach, pharmaceutical brands, and potential future launches provide a solid foundation for sustainable financial success. Viatris's robust pipeline and infrastructure ensure that it will remain a key player in the healthcare industry, poised to deliver value to shareholders while meeting the healthcare needs of millions worldwide.

Mid Cap

Acadia Pharmaceuticals

Acadia Pharmaceuticals, a leading biopharmaceutical company focused on developing medicines to treat central nervous system (CNS) disorders, provided exciting updates at the J.P. Morgan Healthcare Conference. These updates highlight a robust pipeline with the potential to transform the lives of patients suffering from debilitating conditions.

Acadia is projected to generate more than $1 billion in net sales in 2025, the first time in Company history, through the continued success and roll-out of their blockbuster drugs NUPLAZID and DAYBUE.

Today, the European Expansion of DAYBUE was announced, with the company submitting a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for DAYBUE for Rett syndrome. Approval is expected in the first quarter of 2026.

The Phase III COMPASS PWS study of ACP-101, a potential treatment for hyperphagia (excessive hunger) in Prader-Willi Syndrome, is nearing completion. The last patient is expected to be enrolled in the fourth quarter of 2025, with top-line results anticipated in the first half of 2026.

The Phase II RADIANT study of ACP-204, a novel investigational therapy for Alzheimer's disease psychosis, is also progressing well. Patient enrollment is expected to be complete in the first quarter of 2026, with top-line results anticipated in mid-2026. This research could offer a much-needed treatment option for a challenging aspect of this devastating disease. Acadia also plans to initiate a Phase II study in the third quarter of 2025 to investigate its efficacy in treating Lewy Body Dementia.

These updates underscore Acadia's dedication to advancing innovative therapies for patients with serious CNS disorders. With a robust pipeline, a commitment to research, and a focus on unmet medical needs, Acadia is poised to make a significant impact on the lives of many.

Agios Pharmaceuticals

Agios Pharmaceuticals, a leader in cellular metabolism and pyruvate kinase activation therapies for rare diseases, has announced its anticipated key 2025 milestones and value-driving catalysts through 2026. Their currently only rare disease approved asset, Pyrukynd [mitapivat], for pyruvate kinase deficiency, continues to be the near-term backbone of their pipeline programs, with Agios expecting to expand its usage into sickle cell anemia and thalassemia in 2026 and 2025, respectively.

Mitapivat is a pyruvate kinase activator whose mechanism of action increases adenosine triphosphate (ATP) levels, which prevent dehydration and ion loss in sickle red blood cells (RBCs), and decreases 2,3-DPG (2,3 diphosphoglyceric acid), which promotes oxygen unloading, minimizing sickling and hemolysis. Agios took time to reflect on its 2024 highlights, including positive results from the ENERGIZE and ENERGIZE-T Phase 3 trials evaluating mitapivat versus placebo in adults with non-transfusion-dependent and transfusion- dependent alpha- or beta-thalassemia, respectively.

Of commercial note, is the large, currently untreated population for non-transfusion dependent thalassemia, estimated to be approximately 67% of thalassemia patients, representing approximately 4,000 adults in the US alone. Anticipated mitapivat 2025 milestones include receiving FDA regulatory approval for the treatment of adult patients with non-transfusion-dependent and transfusion-dependent alpha- or beta-thalassemia with a PFUDA goal date of September 7 and announcing topline results from the Phase III ACTIVATE-Kids study of mitapivat in children with PK deficiency who are not regularly transfused.

Meanwhile, Agios' development pipeline remains active with the FDA granting orphan drug designation to tebapivat (formerly AG-946) for lower-risk myelodysplastic syndromes (LR-MDS). Following positive Phase IIa results, a Phase IIb trial has been initiated with enrollment expected to be completed by the end of 2025. Additionally, the company is planning to explore this asset in sickle cell disease, similarly to mitapivat, with a Phase II study expected to be initiated in mid-2025.

Development is also occurring in two early-stage assets with an ongoing Phase I study of AG-181, investigating this asset as a PAH stabilizer in phenylketonuria, and an IND expected to be filed for AG-236, a siRNA targeting TMPRSS6 intended for the treatment of polycythemia vera, in mid-2025.

The company remains well funded, with $1.7bn in cash, cash equivalents and marketable securities to further its ongoing commercialization of Pyrukynd and to achieve the ongoing clinical programs.

Ascendis Pharma

Ascendis Pharma, a biopharmaceutical company focused on addressing unmet treatment needs in endocrinology, reiterated their commitment to rapid revenue growth in 2025 at the 43rd JP Morgan Healthcare Conference. The company President and CEO, Jan Mikkelson, began by outlining Ascendis' current position with rare disease medicines Skytrofa (lonapegsomatropin) for pediatric growth hormone deficiency (GHD) and Yorvipath (palopegteriparatide) for adult hypoparathyroidism, both of which have been approved and estimated to have brought in over €220 million in revenues in the full-year 2024. The company's proprietary TransCon technology platform used to develop both drugs forms a huge part of Ascendis' vision for the next five years, with the hope of rolling out several drugs based on the platform in order to expand its endocrinology rare diseases portfolio, obtain accelerated approval in oncology and form significant partnerships with market leaders.

Mikkelson shifted focus to the company's two approved drugs. The first, Skytrofa, was approved in the US in 2021 and confirmed to have been successful in the Japanese Phase III right trial in for pediatric GHD. Revenues generated from Skytrofa sales in 2024 are expected to exceed earnings from the previous year, growing from €179 million in 2023 to about €202 million in 2024, additionally gaining a 6.5% share of the overall US growth hormone market.

Numerous Skytrofa expansion plans were also outlined, including expansion across multiple countries, indication expansion to patients with Turner Syndrome where positive data have already been reported in the Phase II InsiGHTS trial, and an sBLA submission to expand Skytrofa's use to adults by Q3 2025.

Yorvipath's pathway to expansion within the US and EU markets was also detailed, including eight exclusive distribution agreements already signed for over 50 countries, and keen competition with conventional therapy following a successful US launch in December 2024.

Within the company's pipeline, TransCon CNP is the lead asset in development, which met its primary endpoint in the pivotal Phase II ApproaCH trial in pediatric achondroplasia and resulted in quality-of-life improvements and benefits in muscle functionality over placebo with a very tolerable safety profile. In addition to NDA filings anticipated for Q1 2025, results from the COACH trial combining TransCon CNP and Skytrofa for achondroplasia are also expected by the second quarter. With these plans, Ascendis is well-positioned to lead the market in growth disorders in the coming years, as it also targets two other indications – hypochondroplasia and X-linked hypophosphatemia.

Although revenues for 2025 were not projected, Ascendis reported an expected 2024 full-year total revenue of €364 million, including $100 million received to develop a TransCon semaglutide candidate for obesity and type 2 diabetes in a project led by Novo Nordisk. Ascendis has also partnered with Eyconis with a $150 million investor commitment for global exclusive rights for ophthalmology products, Teijin for exclusive rights in Japan and VISEN Pharmaceuticals for exclusive rights in Greater China to market TransCon hGH (Skytrofa), PTH (Yorvipath) and CNP (in development).

These agreements will undoubtedly contribute to revenue growth at the company going forward in 2025.

Avidity Biosciences

Opening remarks reaffirmed Avidity Biosciences commitment to advancing patients' lives through innovative science in rare diseases. The developer has a novel platform, advancing antibody oligonucleotide conjugates, that has successfully delivered RNA to muscle and showed consistent results across three registrational rare neuromuscular programs. These three programs are moving to commercialization in 2025, including Del-zota for Duchenne Muscular Dystrophy (DMD44), Del-desiran for Myotonic Dystrophy Type 1 (DM1), and Del-brax for Facioscapulohumeral Muscular Dystrophy (FSHD). In this way, Avidity Biosciences aims to deliver a new class of RNA therapies, with an emphasis on platform flexibility, novel disease targets, and therapeutic durability.

Time was set aside to discuss the clinical findings for its three most advanced candidates and their addressable patient populations. Firstly, late-stage candidate Del-zota, an asset designed to skip exon 44 of the dystrophin gene to enable dystrophin production, has had robust efficacy and tolerability data reported in August 2024. Treatment with Del-zota showed an up to 66% increase in exon 44 skipping, resulting in a 25% increase in dystrophin production and a restoration of total dystrophin up to 54% of normal.

Del zota has been accepted on the FDA accelerated approval pathway, with a BLA filing anticipated by the end of 2025. This filing will be the first regulatory filing for Avidity Biosciences and marks Del-zota as the most advanced of the three assets. However, this candidate has the smallest treatable population, as it is estimated that approximately 7% of DMD patients are amenable to exon 44 skipping, representing about 900 patients in the US.

Secondly, Del-desiran, an asset designed to address the underlying cause of myotonic dystrophy, has shown positive results of reversing disease progression in its Phase I/II trials, MARINA and MARINA-OLE, with an acceptable safety and tolerability profile. The global Phase III HARBOR confirmatory trial has been initiated (with FDA and EMA guidance) and is on track to complete enrollment in mid-2025. If this late-stage trial is successful, Del-desiran would potentially be the first approved drug for DM1, with a relatively large rare disease population of approximately 80,000 patients in the US and EU.

Lastly, Del-brax is designed to address the root cause of FSHD, the most common form of muscular dystrophy, by directly targeting aberrant expression of homeobox 4 (DUX4) mRNA for destruction. This asset is on track to be the first globally approved drug for FSHD. The Phase I/II FORTITUDE biomarker cohort enrollment is set to be completed in Q2 2025, with all 39 patients enrolled remaining in the study.

In the initial results presented, Del-brax was not only well tolerated but showed a greater than 50% reduction in DUX4-regulated genes across multiple gene panels. This led to improved muscle strength and increased reachable workspace compared to placebo. A Phase III pivotal trial is targeted to initiate in Q2 2025, following results from the FORTITUDE biomarker study. If successful, Del-brax would be serving a similarly sized population to Del-desiran, estimated between 45,000 to 87,000 patients in the US and EU.

The company briefly outlined its expanding reach to precision cardiology and next-generation technology innovations such as siRNA modification and antibody engineering. Other assets discussed include their early-stage cardiology candidates, AOC 1086 and AOC 1072, designed to address PLN cardiomyopathy and PRKAG2 syndrome, respectively. Financially, Avidity remains well funded with a cash runway into mid-2026 of approximately $1.6bn.

BridgeBio

At the 2025 J.P. Morgan Healthcare Conference, BridgeBio provided a detailed update on recent accomplishments and future priorities. The company highlighted its continued clinical progress, including the full enrolment of three Phase 3 trials: BBP-418 for Limb-Girdle Muscular Dystrophy Type 2I/R9 (LGMD2I/R9), encaleret for Autosomal Dominant Hypocalcemia Type 1 (ADH1), and infigratinib for achondroplasia. Regulatory milestones included the FDA’s approval of Attruby (acoramidis) for transthyretin amyloid cardiomyopathy (ATTR-CM), with European and Japanese approvals anticipated in 2025.

BridgeBio emphasised the significant commercial potential of Attruby, targeting a multi-billion-dollar market, with 430 prescriptions written and 248 unique prescribers to date. The company outlined a patient-first market access approach, including simplified reimbursement and patient support programs, to enhance adoption and retention. Attruby demonstrated robust efficacy, showing a 42% reduction in cardiovascular-related hospitalizations and near-complete stabilization of transthyretin levels in clinical studies.

The presentation also underscored the breadth of BridgeBio's pipeline, including pivotal upcoming data for BBP-418 in LGMD2I/R9 and encaleret in ADH1, both expected in late 2025. Additional highlights included promising data for BBP-812 in Canavan disease and further progress in oncology with novel KRAS inhibitors entering the clinic.

Looking ahead, BridgeBio projects continued momentum with approvals, data readouts, and regulatory submissions across key programs. Their strategic focus remains on addressing rare genetic diseases with unmet needs while reinforcing financial stability and operational agility.

Catalyst Pharmaceuticals

Catalyst Pharmaceuticals is a mission-driven biopharmaceutical company dedicated to addressing unmet needs in rare diseases. The company emphasizes health equity and broadening market access to ensure that life-changing therapies can reach the patients that need them most. It offers free bridge medication along with programs that help patients afford treatment even if insurance refuses coverage, demonstrating Catalyst's commitment to care access. The company maintains a strong financial position with $500 million and no debt, enabling these patient access programs as well as supporting life cycle management and the acquisition of new assets.

The company's flagship product, Firdapse, is the only FDA-approved therapy for Lambert-Eaton Myasthenic Syndrome (LEMS). With prescription approval rates exceeding 90% and high patient retention, Firdapse has achieved 30% market penetration in an addressable market valued at over $1 billion, despite the rarity of the indication.

Growth opportunities in this market include label expansions for higher dosing, increasing the patient population by identifying LEMS patients who have been misdiagnosed, and collaborating with oncologists to capture more patients with cancer-associated LEMS. Furthermore, patent protection for Firdapse extends to 2035, offering plenty of opportunity for further growth.

Catalyst has two other drugs, aGamree, a differentiated corticosteroid for Duchenne Muscular Dystrophy (DMD), and Fycompa, an antiepileptic drug that boasts a 72% seizure freedom rate in an adjunctive setting. Since the latter faces imminent generic competition starting in mid-2025, the company has shifted efforts towards aGamree with the initiation of the SUMMIT study in 2024. SUMMIT aims to demonstrate aGamree's clinical superiority in behavior, stature, bone health, and other key outcomes by following ~250 DMD patients for five years. Despite a recent uptick in DMD drug development, steroids will likely remain the first line standard of care, securing space in the market for aGamree, particularly if it can outperform other steroids.

Crinetics

Founded in 2008, Crinetics is focused on developing therapeutics for endocrine related therapeutics. In 2024. Crinetics kicked off its presentation at the 43rd Annual J.P. Morgan Healthcare Conference by reviewing its successes from 2024. In 2024, the Phase III program of paltusotine was completed for acromegaly and an NDA was accepted by the U.S. FDA with a PDUFA assigned for September 25, 2025. A Phase III trial was initiated in carcinoid syndrome after positive Phase II results. Positive Phase II results for atumelnant in congenital adrenal hyperplasia and initial results in Cushing's disease were announced.

In 2025, Crinetics expects four INDS in its next wave of innovation to address unmet needs in large patient populations with an IND submission for CRN09682 planned for NETs along with additional programs including a PTH antagonist for hyperparathyroidism, TSH antagonist for Graves/TED and a SST3 agonist for ADPKD. Further in the pipeline includes an oral GLP-1 nonpeptide and oral GIP nonpeptide programs for obesity with candidate selection expected in 2025.

Pending on approval, Crinetics is commercially ready for paltusotine's launch by building global commercial capabilities to support its endocrinology pipeline. An EU regulatory filing is also expected for paltusotine in the first half of 2025. Crinetics plans to expand even further in 2026 by launching paltusotine in a second indication and the potential launching of atumelnant in two indications and clinical trial catalysts with pivotal data readouts.

Cytokinetics

Robert I. Blum, President and Chief Executive Officer for Cytokinetics highlighted that the company's mission is "to bring forward new medicines that may improve the health span of people with devastating cardiovascular and neuromuscular diseases of impaired muscle function" and that mission is set to make an important step with the launch of its first product expected in 2025. Cytokinetics' leading asset is aficamten, a novel, oral,

small molecule cardiac myosin inhibitor (CMI). The company also has omecamtiv mecarbil, a cardiac myosin activator, for heart failure with reduce ejection fraction (HFrEF) and CK-586, another cardiac myosin inhibitor) for HF with preserved ejection fraction (HFpEF) in clinical development with several other less advanced drug candidates targeting muscle biology.

Over the next 5 years, Cytokinetics is hoping to see the successful commercialization of aficamten and omecamtiv mecarbil focusing on North American and European launches. As a specialty cardiology business Cytokinetics can benefit from various factors improving returns on investment including a targeting a concentrated number of prescribers, the opportunities to grow diagnosed populations, and proving bespoke patient experiences.

The buildup to launching aficamten is underway with the initiation of an HCM awareness campaign and plans for an experienced sales team. The market for CMIs could reach $10 billion across the United States and Europe, according to Blum. Aficamten, if approved for HCM, will be following to market Camzyos (mavacamten), a cardiac myosin inhibitor developed by Bristol Myers Squibb, but it has the potential to become sales leader by growing the market and differentiating itself in terms of efficacy and safety. Data from the SEQUIA-HCM Phase III trial suggested symptomatic benefits based on peak VO2 data and safety benefits including no treatment interruptions due to low LVEF and no episodes of worsening HF. Market access was identified as a critical focus with Cytokinetics looking to build relationships with payers with early engagement and provision of economic and outcomes data as well as supporting patients with dispensing and access assistance. An FDA decision on aficamten is expected by the PDUFA date of 26 September 2025 and regulators in Europe are already considering a marketing application. In Asia, the company is building marketing relationships with Bayer in Japan and Sanofi in China. Key milestones for Cytokinetics in 2025 include data from the Phase III MAPLE-HCM trial of aficamten monotherapy in obstructive HCM and completion of enrollment for ACACIA-HCM, a Phase III trial of aficamten in non-obstructive HCM, and CEDAR-HCM, a pediatric obstructive HCM trial.

Following FDA recommendation, Cytokinetics is evaluating omecamtiv mecarbil in a confirmatory Phase III trial of patient with worsening HF. These patients account for the majority of HF hospitalization with associated costs in the billions each year. With limited therapeutic options, the company is positioning omecamtiv mecarbil to be a new premium-priced alternative for patients with severe HFrEF who do not respond to guideline-directed medical therapy. CK-586 is a CMI being investigated for HFpEF with a Phase II trial planned to complete in 2026. Blum closed the presentation by highlighting the solid financial position of the company with respect to its launch plans and the potential for future R&D investment as revenues from the successful commercialization of new products are realized.

Exelixis

Exelixis's longstanding CEO Michael Morrissey presented the company's progress in building a multi-franchise oncology business and its goal of becoming a market leader in both GU and GI oncology at the 2025 JP Morgan Healthcare Conference. The company reported total revenue of $2.165bn for the fiscal year 2024, with anticipated total revenue of between $2.15bn and $2.25bn for fiscal year 2025. R&D expenses are expected to rise from $910m to between $925m and $975m in 2025. According to Morrissey, this high level of investment is a key strategy to drive the company's mid-term and long-term growth.

Exelixis's only approved drug, the blockbuster TKI Cabometyx, remains the primary value driver for the company, demonstrating strong performance across key commercial metrics due to its competitive clinical data. Since the approval of the Cabometyx and Opdivo combination in 2021, based on the Phase III CheckMATE-9ER trial, this TKI and IO combination has become the most prescribed first-line therapy for RCC. Additionally, Cabometyx leads the second-line RCC market following the success of the Phase III METEOR trial and is approved for patients with HCC and DTC. In Q4 2024, Cabometyx's US net product revenue was $509m, representing a 7% quarter-on-quarter increase and a 19% year-on-year increase. CEO Morrissey emphasized the successful defence of Cabometyx's IP in 2024, which will protect US franchise revenues through 2030 and underpins the company's optimistic growth outlook. Further growth for Cabometyx is expected from the promising Phase III CABINET trial results, which investigated the drug in pNET and epNET tumors. The PDUFA date for this indication is set for April 3, 2025. The CABINET trial featured broad inclusion criteria, including patients who had received Lutathera, a standard-of-care treatment in the pNET and epNET space. If approved, Cabometyx would address a significant unmet need in this patient population and would be the only branded small molecule treatment in the NET space, which has the potential to exceed $4bn in global revenues by 2030. Exelixis is also seeking to expand Cabometyx's label in combination with Roche's Tecentriq for patients with mCRPC, based on the Phase III CONTACT-02 trial. However, Morrissey did not discuss this combination, despite Ipsen, which commercializes Cabometyx outside of the US and Japan, having shelved the combination due to a lack of significant OS data. Based on the discussed value creation, Exelixis projects that Cabometyx will generate $3bn annually in the US by 2023.

The continued uptake of Cabometyx and strategic label expansions are expected to support Exelixis's efforts to advance its differentiated pipeline candidates. The company's next-generation TKI, zanzalintinib, is progressing through a comprehensive development program and has the potential to become a key revenue driver from 2030 onward. Topline results from the Phase III STELLAR-304 trial in non-clear cell RCC and the Phase

III STELLAR-303 trial in CRC are expected in the second half of 2025. Exelixis noted that zanzalintinib could launch as early as 2026 in third-line and beyond CRC if the STELLAR-303 trial is successful. In October 2024, Exelixis entered a strategic clinical development collaboration with Merck to investigate zanzalintinib in combination with Keytruda in a Phase III trial for HNSCC and in combination with Welireg in a Phase I/II trial, as well as two Phase III trials in the clear-cell RCC space. This partnership is expected to sustain Exelixis's momentum in oncology through 2025 and beyond. Morrissey revealed that the company expects zanzalintinib to generate approximately $5bn in US revenue by 2033.

Further long-term growth drivers include advancing three clinical programs — XL309, XB010, and XL485 — and initiating the company's first bispecific program, XB628. In December 2024, Exelixis entered into an exclusive clinical development agreement with Sairopa for ADU-1805, a novel anti-SIRPα antibody, currently in Phase I development. In his closing remarks, Morrissey emphasized Exelixis's continued investment in a diversified pipeline across multiple tumor types to improve the standard of care.

Inspire Medical Systems

Inspire Medical Systems' CEO Tim Herbert CEO kicked off the presentation speaking about the company's mission to enhance patient's lives through sleep innovation. It is focused on the development and commercialization of innovative, minimally invasive solutions for patients with obstructive sleep apnea (OSA), which is an over $10 billion opportunity. The company was founded in 2007 and completed an IPO in 2018. It has treated over 90k patients and has revenue of over $800 million.

Mr. Herbert went on to present fourth quarter and full-year 2024 highlights. There were 72 new US centers activated in Q4, bringing the total centers to 1,435. It created 12 new US sales territories during the quarter, bringing the total to 335 US sales territories. It received FDA approval of the Inspire V neurostimulation system and initiated soft launch with over 40 implants in Singapore and the US. Revenue for full year 2025 is anticipated to be in the range of $940 million to $955 million, a 17% to 19% increase over full year 2024.

Inspire therapy is an innovative and proven solution for patients with OSA. It uses a proprietary closed-loop sensing algorithm to modulate therapy delivery and is performed in a 60-90 minute outpatient procedure, requiring only two small incisions. Patients recover quickly and resume normal activities within a few days. Inspire therapy is the only FDA-approved OSA therapy that works comfortably inside your body. The small Inspire implant delivers gentle pulses to your airway muscles to keep your airway open so you can breathe regularly and sleep soundly. The technology behind the product is continuously evolving. The newest product, Inspire V, is expected to have 20% reduced implant time and improved therapy performance. It is on track for a full launch in 2025.

Mr. Herbert pointed out that continued scaling of the organization provides the opportunity to align and focus its management team for continued growth. The company announced the appointment of Jason Kelly as its new Chief Manufacturing and Quality Officer effective January 20, 2025. Carlton Weatherby will step into the expanded role of Chief Strategy and Growth Officer and lead the US Sales, Marketing, and Strategy teams. Randy Ban will transition from the role of Chief Commercial Officer to a newly created role of EVP, Patient Access and Therapy Development. Ivan Lubogo will transition from the role of SVP US Sales to the newly created role of SVP, Strategic Sales. Joe Sander will be promoted to the role of SVP US Sales. He currently leads the Inspire field team for the Eastern half of the US. Mr. Herbert concluded the presentation saying that the company has the keys to be successful and seeks to continue to invest in top line growth while improving profitability.

Madrigal Therapeutics

At the JP Morgan Healthcare conference, Chief Executive Officer (CEO) Bill Sibold's reaffirmed Madrigal Therapeutics (MDGL) commitment to treating non-alcoholic steatohepatitis (NASH), which is increasingly referred as to as metabolic-associated steatohepatitis (MASH). Accordingly, Madrigal is emerging as the leader in the field of NASH with the landmark US approval of Rezdiffra as the first and only approved NASH therapy in March 2024.

As Madrigal's only approved drug, Rezdiffra is the primary value driver for the company, displaying strong commercial performance across key sales metrics. Upon launch in the US, Rezdiffra netted $14.6 million in the second quarter, $62.2 million in the third quarter, and an estimated range of $100 to $103 million sales in the fourth quarter of 2024. These figures represent an approximately 60% quarter-on-quarter growth, indicating a robust demand for this drug. The company also reported an unaudited full year revenue of between $177 and $180 million for the fiscal year 2024. Overall, Sibold highlighted that the launch for Rezdiffra was exceptional and is in-line with other specialty blockbuster launches despite initial low treatment penetration. Given NASH is an underdiagnosed condition, Sibold noted that there is a lot of room for growth as more competition will drive disease awareness and diagnosis, thus expanding the NASH market.

Madrigal is also exploring Rezdiffra as a treatment for cirrhotic NASH, a condition in which the liver is irreversibly scarred and damaged. The MAESTRO-NASH OUTCOMES Phase III trial is an event-driven trial evaluating Rezdiffra in patients with compensated MASH cirrhosis. The study recently completed enrollment in October 2024, with data expected in 2027. If successful, it could potentially support Rezdiffra's approval in cirrhotic NASH, addressing a significant unmet need in this population as the only approved treatment.

Closing out the presentation, Sibold emphasized that 2024 was a transformational year for Madrigal and the NASH field, starting with the US approval of Rezdiffra as the first and only treatment for NASH. With over 11,800 patients treated as of year-end 2024, Rezdiffra is poised to be a blockbuster success with multiple avenues of growth.

For 2025, Madrigal's strategic priorities include expanding commercial infrastructure in Europe ahead of the anticipated approval for Rezdiffra and establishing it as a foundational NASH therapy globally. Beyond 2025, Madrigal anticipates data from the MAESTRO-NASH OUTCOMES Phase III trial in 2027. The company remains well funded, with approximately $931 million in cash, cash equivalents, restricted cash and marketable securities, to further its ongoing commercialization of Rezdiffra and to continue the ongoing clinical programs.

Novocure

Novocure is an oncology-focused company developing the novel and innovative cancer therapy Tumor Treating Fields (TTFields) for multiple solid tumor types. Novocure's Executive Chairman, William Doyle, remarked that the company, founded over 20 years ago on the principle of using intermediate frequency electric fields to disrupt cancer cell division processes, has built a strong commercial foundation in the treatment of glioblastomas (GBM), making tremendous progress with approvals and reimbursements in major markets, in addition to strong recommendations for use in the US market, over $600 million in revenue recorded globally as of Q1 2025 and more than 4,000 GBM patients so far receiving Novocure's proprietary treatment. Doyle also expanded on the company's three core achievements in 2024, including a growth in GBM business following a successful launch in France and improved US approval and collection rates, a launch in the lung cancer market after a successful Premarket Approval by the FDA and the company's delivery on its development pipeline as it saw significantly positive results from active Phase III trials METIS (NSCLC with brain metastases) and PANOVA-3 (pancreatic cancer).

Mr. Doyle handed the presentation over to the company's newly minted CEO Ashley Cordova, taking over from former long-standing CEO Asaf Danziger, who emphasized Novocure's commitment to its next phase of progress from a single indication company to a multiple indications outfit in three clinically de-risked indications (GBM, pancreatic cancer and NSCLC), all of which have shown poor survival data at five years in patients with these specific tumor types. Novocure has high hopes that the positive showing of its TTFields platform in clinical trials will potentially result in a seven-fold expansion of the company's total addressable market, according to Cordova, as it targets several commercial milestones from its clinical pipeline over the next two years, including primary data from the Phase III TRIDENT trial in GBM for TTFields in combination with temozolamide and radiation and the Phase II PANOVA-4 trial in pancreatic cancer in

combination with Tecentriq and chemotherapy, both expected in 2026, and submissions in 2025 for brain metastases (METIS) and pancreatic cancer (PANOVA-3). Cordova concluded by highlighting that Novocure's vision of extending survival in patients with aggressive cancers will remain the company's focus, driven by potentially increasing revenues from GBM, $213 million in clinical and product R&D investments and its intellectual property in the short term, with an eye towards advancements into label-expanding trials in the future.

Nuvalent

James Porter, CEO of Nuvalent, Inc., led this year's JPM discussion which centered on providing updates on their major drug development projects namely, zidesamtinib (NVL-520), neladalkib (NVL-655), and NVL-330 for ROS1+, ALK+ and HER-2 altered NSCLC respectively. He indicated that both the ROS1-positive and ALK-positive NSCLC markets have high commercial potential with $3.3 billion combined worldwide sales in 2023. The company is aiming to disrupt the current treatment paradigms in both these therapeutic areas with best-in class therapies, namely zidesamtinib, neladalkib that have already achieved FDA Breakthrough designations. The pivotal clinical data-outs for both these programs, ARROS-1 and ALKOVE-1 are expected in 2025. Also, the company plans to initiate a Phase III trial, ALKAZAR in the first half of 2025 to establish neladalkib as a potential best-in-class drug for the fist-line treatment of patients with ALK-positive NSCLC. Additionally, the company has also initiated HEROEX-1, its Phase 1a/1b clinical trial evaluating its novel HER2-selective inhibitor, NVL-330, for pre-treated patients with HER2-altered NSCLC.

He also briefly reflected on their 2024 milestones highlighting the completion of important trial phases, and delineated strategic objectives for 2025, which included aiming for its first potential approval in 2026.

Thus, these developments underscore Nuvalent's dedication to addressing the limitations of existing cancer therapies by creating precisely targeted treatments designed to overcome resistance, minimize adverse events, and address brain metastases.

Recursion

Chris Gibson, Co-Founder and CEO of Recursion kicked off his JPM presentation with an overview of the company's mission of decoding biology to radically improve patients' lives. The full-stack Recursion Operating System (OS) platform is transforming drug discovery and translation based on real-world data and machine learning to simulate biology and pathways at the cellular level. Recursion's recent business combination with Exscientia creates a unified OS and an expanded technology-enabled portfolio with more than 10 clinical and preclinical programs, 10 advanced discovery programs, and more

than 10 partnered programs in oncology, rare diseases, and other high unmet need diseases. Dr. Gibson highlighted a robust oncology portfolio which includes REC-1245, a highly selective potential first-in-class RBM39 degrader for biomarker-enriched solid tumors and lymphoma. The R&D process took only 18 months from target identification to IND-enabling studies. The first patient was dosed in the Phase I dose-escalation study in Q4 2024, with an update expected in 1H2026. Recursion's rapid design cycle synthesized 136 novel compounds under 11 months from hit to candidate ID including precision designed CDK7 inhibitor REC-617. Early data indicates a favorable safety profile of the durable monotherapy in a metastatic ovarian cancer patient after 4 prior lines of therapy. The company plans to initiate a combination study in 1H2025. Recursion's rare disease pipeline includes REC-994, a safe and well tolerated superoxide scavenger for the treatment of Cerebral Cavernous Malformation (CCM). No medical treatment currently exists for CCM, but REC-994 met its primary endpoint of safety and tolerability in its Phase 2 study, with full results to be presented at ISC on February 5, 2025. Recursion is also developing a potential first-in-class non-antibiotic oral toxin B selective inhibitor for the prevention of recurrent C. difficile (rCDI), REC-3964. Recruitment is ongoing with a Phase 2 update expected in Q1 2026.

Recursion has four large pharma collaborations with Roche/Genentech (neuroscience and oncology), Bayer (oncology), Sanofi (oncology and immunology) and Merck KGaA (oncology and immunology). To date, the partnerships have earned $450M in upfront and milestone payments, with the potential for up to $20B in additional milestones. Announced today, as part of the Roche partnership, is the world's first neuro-specific wholegenome arrayed CRISPR KO phenomap optioned for $30M.

In closing, Dr. Gibson provided Recursion's major business updates. The company is deeply focused on maximizing the return on the business combination with Exscientia. The senior-leadership teams have combined, and the integration of EXAI precision design platform into Recursion OS is underway. A 90-day goals update will be given at YE24 earnings, and guidance on combination synergies will be presented in Q2 2025. As of September 30, 2024, pro forma for the combined entity, cash and cash equivalents is $752 million.

Roivant Pharma

Matt Gline, Roivant's CEO, began the company's presentation today by expressing their excitement for the coming year within the company. Gline provided an overview of the transformational potential of the company for 2025. Three main points of this potential are: opportunity to validate first/best-in-class anti-FcRn potential, potentially registrational DM readout sets stage for commercial launch of Brepocitinib, and advance LNP litigation with Moderna and Pfizer/BioNTech.

Gline then went on to cover their lead candidate IMVT-1402, a novel, fully human, monoclonal antibody inhibiting FcRn-mediated recycling of IgG. The company plans to be in 10 indications with this candidate in a year from now. Initial Phase 1 data suggests deep dose-dependent IgG lowering. IMVT-1402 also showed a clean safety profile. Gline expressed that the most promising indication of this drug currently is Graves' disease. Data in this indication demonstrated transformational results in patients uncontrolled on ATDs with a greater response driven by deeper IgG lowering. With such a high unmet need in this indication, Roivant believes they are in a good position to meet this need with IMVT-1402.

Gline then moved on to their potential first-in-class dual selective TYK2/JAK1 inhibitor, Brepocitinib. Brepocitinib combines the best attributes of selective TYK2 and JAK1 inhibition with potential to provide maximum efficacy for patients with highly morbid, heterogeneous autoimmune diseases. Clinical experience over seven positive Phase 2 studies suggest oral Brepocitinib is highly active and able to generate clinical benefit in TYK2-and JAK1-driven indications. Gline also discussed the active LNP litigation with Moderna and Pfizer/BioNTech with the upcoming jury trial in September 2025.

In an overview of Roivant's late-stage pipeline, Gline discussed the expectations for the upcoming year and the excitement in the company for what is to come. With multiple potentially registrational trials, Roivant is confident in their ability to successfully accomplish their goals for the upcoming year. In 2026 and beyond, the company expects readouts from multiple late-stage potential blockbuster opportunities from 7 programs initiated in 2024, including IMVT-1402, Brepocitinib, and Mosliciguat. In terms of the company's overall cash balance, they believe they are in a strong capital position to push towards the aforementioned goals. Gline closed out the presentation by stating that the company has ongoing business development with multiple ongoing negotiations for potential in-licensing of new programs.

Telix Pharmaceutical

Telix Pharmaceuticals' newly appointed EO, Chris Behrenbruch, opened the company's presentation at the 2025 JP Morgan Healthcare Conference by outlining their specialist approach in the theranostic radiopharmaceutical oncology space. Behrenbruch emphasized that Telix's theranostic strategy is central to the company's vision and a key driver of value. The company has demonstrated strong year-over-year revenue growth, achieving a 55% increase between Q4 2023 and Q4 2024, with US revenue reaching $142m. The CEO also highlighted that the indications Telix are pursuing offer significant long-term growth potential and that the company is uniquely positioned to fund the majority of its R&D through earnings, an uncommon feat for a company at such an early stage.

Telix's diagnostic medicine business serves as both the company's primary revenue generator and the market entry point for its therapeutic assets. The US sales of Illuccix, its only approved diagnostic drug for prostate cancer patients, were the main driver of this growth, with a 64% increase in revenue compared to H1 2023, as reported in the company's Q4 2024 financial results. The continued uptake of Illuccix is partially attributed to a strategic label expansion, allowing its use in select US patients who are candidates for Pluvicto, a standard-of-care prostate cancer therapy. Further global expansion is expected in 2025, with regulatory approvals anticipated in the EU, UK, and Brazil. Additionally, Phase III trials have commenced in Japanese- and Chinese-only populations. Behrenbruch also discussed upcoming US product launches in 2025. Gozellix, an imaging agent for prostate cancer, has a PDUFA date for March 2025, while Pixclara, an imaging agent for glioblastoma, has a PDUFA date set for April 2025. Zircaix is also expected to launch in 2025 for clear-cell RCC; however, its regulatory process has been delayed following a BLA resubmission in January 2025 prompted by FDA concerns about sterility assurance. Importantly, the concerns were not related to the agent's safety or efficacy, and a new PDUFA date is expected in August 2025.

Telix outlined its therapeutic strategy roadmap for its beta-emitting radiopharmaceuticals, which have multiple near-term catalysts. The company's key late-phase therapeutic asset, TLX591, demonstrated strong efficacy in the Phase I ProstACT SELECT trial, with an interim readout of the Phase III ProstACT GLOBAL trial expected in H1 2025. A notable advantage of TLX591 is its highly condensed dosing schedule, which integrates well with the current standard-of-care. Additionally, the Phase II STARLITE 2 trial, investigating the combination of TLX250 and Opdivo in clear-cell RCC patients, is expected to report results in February 2025. Meanwhile, TLX101 is being evaluated in the Phase I IPAX-2 trial for first-line glioblastoma following positive FDA feedback on trial design, and the Phase II IPAX-L trial in the recurrent setting has completed enrolment, with topline data expected in H1 2025. Telix are also developing a robust pipeline of early-phase, next-generation alpha-emitting radiopharmaceuticals — TLX592, TLX252, TLX102, and TLX300 — which hold significant long-term growth potential.

To further enhance its clinical expertise, Telix has entered into asset purchase and exclusive worldwide in-licensing agreements for a suite of clinically validated FAP-theranostic radiopharmaceutical candidates developed at the Institute of Nuclear Chemistry at Johannes Gutenberg-Universität Mainz. Notably, the next-generation asset TLX400 is being investigated for its theranostic application in thyroid cancer patients. Additionally, in May 2024, Telix partnered with QDOSE® and acquired QSAM Biosciences in a deal worth up to $125m, gaining access to Samarium-153-DOTMP for metastatic bone cancer. The company is also strengthening its supply chain and manufacturing capabilities through acquisitions of ARTMS1 and IsoTherapeutics, as well as investments in R&D facilities, to overcome barriers to market uptake.

In closing remarks, Behrenbruch underscored Telix's focus on patient access and product life-cycle management. He expressed confidence that Telix's market share will grow significantly in the short term, driven by its highly competitive clinical and commercial strategy.

Vericel

Presenting on behalf of Vericel at this year's JPM conference was president and CEO Nick Colangelo. Vericel is a leader provider of advanced therapies in sports medicine and severe burn care, combining innovations in biology with medical technologies. It has a portfolio of innovative cell therapies and specialty biologics with significant barriers to entry. The company is well positioned to deliver sustained long-term growth.

Vericel's sports medicine portfolio is led by MACI, which is an autologous cellularized scaffold product indicated for the repair of cartilage defects of the knee in adults. MACI is the leading restorative knee cartilage repair product on the market. MACI Arthro is the first restorative biologic cartilage repair product approved for arthroscopic administration. MACI Arthro enables additional surgeon growth and is expected to drive incremental volume across all surgeon segments. The product launched in Q3 2024 and there is continued strong growth in MACI surgeons and biopsies. Vericel is expanding its core portfolio and is on track to submit an IND for MACI Ankle in H1 2025 and initiate a clinical study in H2 2025. If approved it would be the only product available for ankle cartilage repair, which represents a $1 billion market opportunity. A new manufacturing facility provides flexibility to potentially commercialize MACI outside the US.

Leading its burn care franchise is the Epicel skin graft for the treatment of patients with deep dermal or full thickness burns greater than or equal to 30% of total body surface area. It is the only FDA-approved permanent skin replacement for patients with severe burns and is an important treatment option for severe burn patients where little skin is available for autografts. There are no competitors on the horizon for MACI or Epicel.

Vericel also sells NexoBrid, a biological orphan product containing proteolytic enzymes that is indicated for eschar removal in adults and pediatric patients with severe burns. NexoBrid is a non-surgical topical agent that may be applied at the patient's bedside. It selectively degrades eschar in four hours while preserving viable tissue.

Yesterday Vericel announced preliminary 2024 financial results. It has a strong financial position with high revenue growth profile and strong balance sheet. It has sustained positive adjusted EBITDA and operating cash flow. Total net revenue is expected to be approximately $237 to $237.5 million, representing 20% growth. MACI net revenue is anticipated to be approximately $197.2 to $197.7 million, also representing 20% growth. The burn care net revenue is expected to be approximately $40 million,

representing 22% growth and consists of approximately $36.6 million of Epicel revenue and $3.3 million of NexoBrid revenue. Gross margin is expected to be approximately 72.5% for 2024. Vericel achieved full-year GAAP net income profitability. As of December 31, 2024, the company had approximately $167 million in cash, restricted cash and investments, and no debt, an increase of approximately $16 million for the quarter. Vericel comes into 2025 with great momentum on the heels of a strong 2024 and expects to have total growth of 20-23% in 2025.

Viking Therapeutics

Viking Therapeutics (VKTX), led by Chief Executive Officer (CEO) Brian Lian, is a biopharmaceutical company that focuses on the development of novel therapeutics for metabolic and endocrine disorders. Dr. Lian began the presentation by outlining Viking's

current progress on two metabolic indications: obesity and non-alcoholic steatohepatitis (NASH), which is increasingly referred to as metabolic-associated steatohepatitis (MASH). Since its inception, the company has developed multiple clinical programs. However, given that Viking currently has no late-stage products, the company is entering a critical moment to transition from a mid-stage biotech to a late-stage biopharmaceutical company with a planned Phase III program for subcutaneous VK2735.

Viking Therapeutics' flagship program is VK2735, a dual glucagon-like peptide 1 (GLP-1) and glucose-dependent insulinotropic polypeptide (GIP) receptor agonist. It is being developed in both subcutaneous and oral formulations to treat obesity. VK2735 has demonstrated significant body weight reduction after 13 weeks of treatment. Furthermore, progressive weight loss was observed in all VK2735 dosing cohorts. On safety, adverse effects were mostly mild with minimal early discontinuations, which could differentiate VK2735 from its competitors. Given the positive data, Viking Therapeutics met with the FDA in late 2024 and subsequently are preparing a Phase III program to be initiated in the first half of 2025. If successful, subcutaneous VK2735 is poised to be the company's first commercial product. In addition to the subcutaneous formulation, Viking is also advancing VK2735 in its oral form. Dr. Lian highlighted the initiation of its Phase II VENTURE-Oral study for oral VK2735 in obese patients, announced ahead of its JPM presentation. The initiation was supported by the encouraging data from the Phase I proof-of-concept study of oral VK2735 in obese patients, which showed VK2735-treated patients lost up to 8.2% body weight after 28 days of treatment. Moreover, oral VK2735 demonstrated excellent tolerability profile through 100mg dose level with 99% of adverse events remaining mild and/or moderate with low rates of vomiting, diarrhea and constipation.

In addition, the company also develops VK2809, a thyroid receptor hormone-beta (THR-β) agonist, for NASH and VK0214, also a THR-β agonist but for X-linked adrenoleukodystrophy (X-ALD), a rare genetic disorder affecting the nervous system.

These programs have produced encouraging results, with VK2809 treatment achieved NASH resolution in up to 75% of patients and fibrosis improvement in up to 60% of patients in the Phase II VOYAGE study, while VK0214 showed significant reductions of plasma levels of very long chain fatty acids (VLCFAs), the biomarkers of disease in patients with X-ALD.

In closing remarks, Dr. Lian accentuated further clinical development for the VK2735 programs, with a Phase III program for subcutaneous VK2735 to be initiated in the first half of 2025 and ongoing Phase II clinical works for oral VK2735 with completion expected in the second half of 2025. Furthermore, Dr. Lian noted that the current cash and ST investments of over $900 million is sufficient to sustain operations through Phase III activities and clinical operations. Looking forward, Viking is well-positioned to sustain

its momentum and solidify its standing in the rapidly changing obesity market. With strong financials and a robust pipeline, Viking Therapeutics presents exciting opportunities for investors and patients.

Xenon

Xenon Pharmaceuticals, led by President and CEO Ian Mortimer, is a biopharmaceutical company specializing in the development of innovative therapeutics for neurological and psychiatric disorders. The company's primary focus is ion channel modulators, aiming to provide novel treatments for conditions such as epilepsy and major depressive disorder (MDD). The company also hopes to expand into other neurology indications with its preclinical program exploring other ion channel targets.

Xenon’s only clinical-stage asset is azetukalner, a selective Kv7 potassium channel opener currently Phase III development for multiple indications. In epilepsy, azetukalner is being evaluated initially for focal onset seizures (FOS), with the intention of later expanding into primary generalized tonic-clonic (PGTC) seizures. The ongoing Phase III X-TOLE 2 and 3 studies mirror the design of the successful Phase II study in FOS, which showed some of the largest placebo-adjusted efficacy data ever seen in epilepsy clinical research. Additionally, the X-TOLE open-label extension study has accumulated over 600 patient-years of data, demonstrating sustained reductions in seizure frequency and impressive seizure freedom rates. Notably, 32.7% of patients have experienced 12 or more consecutive months without seizures after 36 months on the drug, a significant achievement given their baseline severity. Additionally, Xenon has initiated the X-NOVA2 trial, one of three planned Phase 3 studies evaluating azetukalner as a monotherapy in

patients with MDD. Depression is a common comorbidity in epilepsy that is often overlooked and even exacerbated by some antiseizure medications. This mechanism is novel in depression as well, offering variety in a disease area with a relatively homogenous treatment landscape. The safety and tolerability profile for azetukalner has been well-characterized and found to be consistent across indications, although lower rates of adverse events were seen in the MDD population.

Looking ahead, Xenon anticipates several key milestones in 2025. The company expects to release topline data from its first Phase III study of azetukalner in FOS in the second half of the year. If these data are consistent with the Phase II results, the company could begin assembling an New Drug Application (NDA) using both the Phase II and Phase III trials. Topline results from an investigator-led study at Mount Sinai evaluating azetukalner in MDD are also expected in the first half of 2025, providing early insight into azetukalner's efficacy in MDD. Furthermore, Xenon plans to file multiple Investigational New Drug (IND) applications for pipeline candidates targeting Kv7, Nav1.7, and Nav1.1 ion channels that showed preclinical promise across multiple neuropsychiatric indications.

Small Cap

AbCellera Biologics

Carl Hansen, CEO, Chairman, and President of AbCellera, kicked off the JP Morgan presentation by highlighting the critical role strategic partnerships have played in driving the company's growth and generating a portfolio of future royalty-bearing antibody medicines. While AbCellera has significantly benefited from these collaborations, Hansen noted a strategic shift in the company's business model. The focus is now moving away from new partnerships in favor of allocating capital toward the development of internal assets. However, Hansen emphasized one ongoing collaboration — a recently expanded agreement with AbbVie, focused on discovering T cell engagers (TCEs) for oncology. Under this partnership, AbCellera will lead discovery efforts, while AbbVie retains the rights to develop and commercialize resulting therapeutic antibodies. AbCellera will receive upfront and research payments, with additional milestones and tiered royalties tied to net sales. These partnerships, coupled with revenues from their work with Eli Lilly on COVID treatments and committed government funding, have provided AbCellera the liquidity and the potential to build a promising pipeline of first-in-class and best-in-class therapeutics.

Looking ahead to 2025, Hansen outlined AbCellera's strategy to transition from a preclinical entity to a clinical-stage biotechnology company. The company evaluates potential opportunities based on high return-on-investment potential, scientific merit, clear commercial viability, and a defined development path. These criteria were pivotal in selecting their two preclinical candidates, ABCL575 and ABCL635, which are expected to enter the clinic later this year. AbCellera plans to continue expanding its pipeline with 1-3 IND filings annually over the next five years, aiming to establish a strong clinical presence. With more than 20 preclinical programs in early development, AbCellera is positioning itself to become a clinical-stage leader with a robust portfolio of candidates that target unmet needs and offer the potential for substantial returns.

In summary, AbCellera is poised for a significant transformation, focusing on the internal development of novel antibody therapies while maintaining strategic partnerships that drive both innovation and revenue. With a clear path to clinical-stage development, Hansen emphasized the company's commitment to advancing its pipeline and meeting the growing demand for differentiated, high-impact medicines.

Akero Therapeutics

Akero Therapeutics, Inc. (AKRO) President & CEO Andrew Cheng, MD, PhD, opened the presentation with an overview of recent progress and near-term milestones for efruxifermin (EFX), a potential best-in-class treatment for metabolic dysfunction-associated steatohepatitis (MASH), which the company originally in-licensed from Amgen through a 2018 deal.

A detailed overview of trial designs and progress was given for the two 96-week Phase IIb clinical trials, HARMONY and SYMMETRY. HARMONY, the first Phase IIb study evaluated the efficacy and safety of EFX in patients with pre-cirrhotic MASH, fibrosis Stage Two or Three (F2-F3). The data demonstrated a substantial improvement in fibrosis between 24 and 96 weeks (for patients treated with 50mg EFX) and no worsening of MASH. The two-stage fibrosis improvement showed the benefits of longer dosing and that the compound's antifibrotic effects don't plateau and can be maintained at 96 weeks. For the SYMMETRY study, the second and ongoing Phase IIb study in patients with compensated cirrhosis due to MASH with Stage Four fibrosis (F4), data at week 36 showed statistically significant fibrosis improvement without worsening of MASH, with four patients experiencing a three- or two-stage fibrosis improvement. Additional evidence of EFX's potential to reverse fibrosis with multiple non-invasive markers of liver health were observed and a case study of fibrosis improvement and MASH resolution was presented. The week 96 readout with liver histology is expected in February 2025.

Building on the strong results from the Phase IIb studies, the Phase III SYNCHRONY

program consists of two efficacy and safety studies with histology and long-term clinical outcomes endpoints and a third one-year study evaluating safety and tolerability. SYNCHRONY Histology (treatment duration 96 weeks; 28mg and 50 mg doses) aims to evaluate EFX in patients with biopsy-confirmed pre-cirrhotic MASH (F2-F3) with a primary histology endpoint of a one-stage fibrosis improvement and MASH resolution at week 52, with a primary outcomes endpoint at week 240. The study readout is expected in the first half of 2027. The SYNCHRONY Outcomes (treatment duration of five years; 50 mg dose) is evaluating EFX for compensated cirrhosis due to MASH (F4), with a primary histology endpoint at week 96.

Cheng highlighted the company's announcement yesterday of the completion of enrollment of 601 patients in the double-blind portion of the SYNCHRONY Real-World study (once-weekly subcutaneous dosing of either 50mg EFX or placebo) in patients with non-invasively diagnosed MASH (F1-F4) or metabolic dysfunction-associated steatotic liver disease. SYNCHRONY Real-World includes an open-label cohort of patients previously assigned to placebo in the HARMONY or SYMMETRY studies. The blinded portion of the study has a primary endpoint of assessing safety and tolerability of EFX during 52 weeks of treatment, and secondary endpoints include changes from baseline in non-invasive markers of liver fibrosis and liver injury, as well as lipoproteins, glycemic control, and body weight. Data from this study is anticipated in the first half of 2026. Akero has cash on hand of approximately $787 million to fund it into the second half of 2027, including seeing the Phase III SYNCHRONY Histology and Real World studies through primary endpoints.

Anavex Life Sciences

Anavex Life Sciences is dedicated to the therapeutic discovery and development of targeted central nervous system (CNS) treatments aimed at upstream cascades or processes to counter neurodegeneration and restore neuronal homeostasis. With a strong emphasis on Alzheimer's disease (AD), Parkinson's disease (PD), Rett syndrome, and other rare CNS conditions, Anavex aims to address significant unmet medical needs globally. In this presentation, CEO Christopher Missling reported $132.2 million in cash and cash equivalents, no debt and a cash runway of four years. Cash utilization in fiscal year 2024 totaled $39.8 million. Anavex aims to bring its lead therapies to patients in Europe, Asia-Pacific, and the US, following regulatory discussions, and is examining innovative strategies to effectively engage patients, providers and payers. Its products possess wide international product protection until 2030-2039.

The company is currently meeting with regulatory bodies around the world to discuss the potentially pivotal Phase IIb/III AD-004 trial evaluating its lead asset, ANAVEX 2-73 (blarcamesine), an oral pill for AD. The drug aims to restore neuronal homeostasis via activation of the SIGMAR1 receptor, a novel target in this indication. In chronic CNS

pathologies, the body's SIGMAR1 activators are exhausted, impairing response to chronic cellular stress. The target binding affinity of ANAVEX 2-73 is so specific that even when patients carry a variant receptor, powerful effects are still observed.

ANAVEX 2-73 has demonstrated superior clinical safety and efficacy compared to marketed monoclonal antibodies, Leqembi and Kisunla, and demonstrated an ability to slow neurodegeneration in early AD. There was significant slowing of atrophy in the broad brain regions after 48 weeks of treatment in the AD-004 trial. Data from the Phase IIb/III ATTENTION-AD open-label extension (OLE), which followed the AD-004 trial, were also recently released, showing continued benefit over three years. A delayed-start analysis of both trials suggested that earlier treatment initiation of ANAVEX 2-73 may have continued long-term beneficial therapeutic effect. The drug has now received EMA filing acceptance for the treatment of AD. ANAVEX 2-73 is also being evaluated in PD and PD dementia.

An oral liquid formulation of ANAVEX 2-73 is being evaluated in Rett syndrome, fragile X syndrome, infantile spasms and Angelman syndrome. Data from the Phase II/III EXCELLENCE trial assessing the drug in pediatric Rett syndrome patients were released in January 2024. Unfortunately, the trial missed the primary endpoint, but >91% of the patients continued into the ongoing 48-week OLE study. Of the pediatric patients who completed the OLE, 93% have joined the Compassionate Use Program. The company has received positive real-world feedback regarding ANAVEX 2-73 in Rett syndrome and some patients have been taking the drug for over four years.

A different asset, ANAVEX 3-71, is being assessed in schizophrenia, frontotemporal dementia and AD. A Phase II trial evaluating ANAVEX 3-71 in schizophrenia is ongoing, with the preliminary data, released in October 2024, demonstrating a dose-dependent effect and evidence of CNS target engagement.

Looking forward, analysis of RNA sequencing from the Phase IIb/III data for ANAVEX 2-73 in AD are expected in 2025, as are top-line data from the Phase II trial assessing ANAVEX 3-71 in schizophrenia. In the near term, we can expect the initiation of an imaging-focused trial or a Phase IIb/III trial longer than six months for ANAVEX 2-73 in Parkinson's disease. The initiation of Phase II/III trials for ANAVEX 2-73 in fragile x syndrome and a new rare disease are also planned. In the longer-term, the company hopes to expand the focus to further CNS indications, regenerative medicine and disease prevention.

Cogent Biosciences

Cogent Biosciences is a company developing focused on development therapeutics for genetically defined diseases. Chief Executive Officer and President Andrew Robbins led the presentation which began with outlining the company's current pipeline, moving on

to report the data readouts that can be expected during 2025, and rounded off by describing the clinical successes that the company's lead compound bezuclastinib has had so far. The company has also reported a strong balance sheet with funds of $345.5 million as of September 30, 2024.

Cogent currently has a total of five ongoing research programmes. Its lead compound bezuclastinib is a selective and potent KIT inhibitor currently being investigated in three ongoing trials, while its FGR2 inhibitor CGT-4859 is in early stage development. Cogent also has an additional three compounds currently at the research stage. Bezuclastinib is in development for three different indications; nonadvanced systemic mastocytosis (NonAdvSM), gastrointestinal stromal tumours (GIST) and advanced systemic mastocytosis (AdvSM) and is currently in an ongoing, pivotal trial for each, with the Phase II SUMMIT trial, Phase III PEAK trial and Phase II APEX trial, respectively. All three trials anticipate data readouts during 2025, with the SUMMIT trial expected to release topline results in July 2025, while topline results for APEX are anticipated during the second half of the year, and topline results from PEAK are expected by the end of 2025.

Cogent is confident in the commercial prospects for bezuclastinib, which it claims has the potential to become a best-in-class KIT inhibitor. So far, in Part 1 of the SUMMIT trial, efficacy results reported that bezuclastinib reduced total symptom score (TSS) by a mean of 27.6 points, with 30% TSS reduction achieved by 88% of NonAdvSM patients. Meanwhile, there is a significant unmet need for GIST therapies in the second line of treatment, which Cogent is investigating through the combination of bezuclastinib + sunitinib in the PEAK trial. Results from Part 1 of the trial showed that the combination produced efficacy results of 40% objective response rate (ORR) with durability, while the safety and tolerability of the combination appears to be generally consistent with the profile for sunitinib monotherapy. Results from Part 1 of the APEX trial were also presented, in which bezuclastinib showed impressive clinical activity in AdvSM patients. The progression-free survival (PFS) rate reported was 82% at 24 months and bezuclastinib demonstrated an encouraging safety profile, with no intracranial bleeding events being reported while the majority of hematological AEs were low grade and reversible and did not require dose reduction. Robbins rounded off the presentation by describing that the aggregate US annual sales potential across all three indications is a total of over £3 billion.

Edgewise Therapeutics

Edgewise Therapeutics (EWTX) CEO Kevin Koch, PhD, opened the presentation with an overview of the company's 2024 accomplishments, including detailed updates on its muscle disease and cardiovascular disease pipeline and related trial progress.

Edgewise is optimistic about the potential of sevasemten, its orally administered first-in-class fast skeletal myosin inhibitor designed to protect against contraction-induced muscle damage in in both Becker and Duchenne muscular dystrophies (MD). Edgewise achieved Fast Track designation for sevasemten for Duchenne from the FDA and Orphan Drug designations for both Becker and Duchenne from the EMA.

In Becker, positive topline data was reported from the CANYON Phase II placebo-controlled trial; end of Phase II feedback from the US FDA is anticipated in the first half of 2025. The GRAND CANYON global pivotal cohort of sevasemten in adults with Becker was substantially enrolled, driving toward over enrollment in Q1 2025. Positive data from this cohort, on track for Q4 2026, could support a marketing application. Positive two-year results from the ARCH open label, single-center study demonstrated stabilization of function with trends toward improvement at 24 months. Positive DUNE trial data also showed sevasemten prevents muscle damage following exercise challenge.

In Duchenne, the company advanced the LYNX Phase II trial, a two-part, dose-finding trial to evaluate the effect of sevasemten in children aged 4- 9 years, as well as the FOX Phase II trial in children and adolescents aged 6-17 previously treated with gene therapy. Full LYNX and FOX data is anticipated in Q2 2025. With 5 mg and 10 mg doses identified, a Phase III Duchenne trial initiation is expected in the second half of 2025.

Much emphasis was also given to the cardiovascular programs, led by EDG-7500, Edgewise's oral, selective, cardiac sarcomere modulator, designed to slow early contraction velocity and address impaired cardiac relaxation associated with hypertrophic cardiomyopathy (HCM), and other diseases of diastolic dysfunction. The company reported positive topline data from both the Phase I healthy volunteer study and from the single-dose arm of the Phase II CIRRUS trial in obstructive hypertrophic cardiomyopathy (oHCM). Phase II 28-day and 12-week trials in oHCM and non-obstructive HCM were initiated. Initial CIRRUS 28-day data can be expected in Q1 2025; data from the 12-week CIRRUS is anticipated by the second half of 2025. The company also anticipates filing an investigational new drug application for an undisclosed second-generation heart failure candidate during the first half of 2025, as well as the selection of a proprietary cardiometabolic drug candidate from preclinical proof-of-concept data by the second half of this year.

Edgewise also highlighted its strong balance sheet, with a $240 million public follow-on public offering in January 2024, giving the company a $493M runway through 2027. In closing remarks, Koch is looking forward to a promising 2025, with multiple value-generating milestones in the company's Becker and Duchenne MD programs, and additional, longer-term data across HCM populations for EDG-7500.

Immunocore

Dr. Bahija Jallal, CEO of Immunocore, introduced the company as a pioneer in T cell receptor antibody development, highlighting its three late-stage pipelines currently in Phase III trials. For 2025, Immunocore's strategic priorities include maximizing the potential of KIMMTRAK, advancing its clinical portfolio, and driving innovation to ensure sustainable growth.

KIMMTRAK has been launched in 23 countries and approved in 38 countries, achieving 65% penetration in the US. It generated $226 million in the first three quarters of 2024, and this product is expected to grow due to further market penetration and increased treatment duration for more than 12 months. The company is conducting two Phase III trials, TEBE-AM and ATOM, which could potentially add 6,000 patients to the treatment pool. The TEBE-AM study aims to position KIMMTRAK as a monotherapy or in combination with an anti-PD-1 drug in the second line or later in cutaneous melanoma, with expected enrollment completion in the first half of 2026 and data availability in the second half of 2026. If approved, it could address 4,000 patients. The ATOM study focuses on high-risk adjuvant uveal melanoma, with approximately 1,200 patients eligible for treatment if approved.

Immunocore is advancing its PRAME portfolio with brenetafusp in three trials: cutaneous melanoma (PRISM-MEL-301), ovarian cancer, and non-small cell lung cancer. PRAME is expressed in multiple solid tumors and is a negative prognostic marker. The PRISM-MEL-301 study is a global Phase III trial to test brenetafusp as a first-line therapy combined with nivolumab, with an estimated 10,000 addressable patients. Immunocore plans to conduct trials in platinum-resistant ovarian cancer in combination with non-platinum chemotherapy and move brenetafusp to an earlier line of treatment in platinum-sensitive ovarian cancer in combination with standard of care treatment, with data expected in 12-18 months. Currently the trial in NSCLC is still in the middle of signal searching.

The company is also exploring PIWIL1 as a target for colorectal and GI cancers. PIWIL1 was found to be highly expressed in CRC, and cancers expressing PIWIL1 are insensitive to immunotherapy. The Phase I study was launched in Q4 2024 with data expected in 12-18 months. Additionally, Immunocore is developing an HIV treatment targeting the viral reservoir in hopes of preventing HIV rebound after anti-viral agent cessation. In this dose-finding Phase I study, the planned highest dose at 300 mg has not reached the dose-limited toxicity so an amended protocol that allows testing higher doses has been cleared. The preliminary data from 15 patients are anticipated in Q1 2025. Additionally, Immunocore is developing its tissue-tethered antibody for autoimmune diseases with organ specificity. The antibody contains an Fc fusion domain to extend antibody half-life and an PD1 agonist domain to suppress T cell activity. The company plans to apply such an antibody (dubbed IMC-S118AI) in newly diagnosed Type 1 diabetic patients to

prevent their beta cells from immune attack. The CTA submission of IMC-S118AI for a Phase I study is expected by the end of 2025. They are also developing a first universal candidate targeting CD1a (dubbed IMC-U120AI) for treating atopic dermatitis.

In the next 12-18 months, Immunocore plans to expand KIMMTRAK's leadership in mUM and label expansion with TEBE-AM and ATOM studies. The company will also progress the PRAME portfolio in Phase III PRISM-MEL-310 and two Phase II trials for ovarian and NSCLC, as well as the HIV multiple-ascending dose study. Two CTA filings are anticipated in the next 18 months. The company has preliminary unaudited cash, cash equivalents, and marketable securities of $820 million as of the end of 2024.

Intellia

Just days after announcing a strategic reprioritization involving laying off over a quarter of the company's workforce, Intellia Therapeutics' President and CEO, John Leonard, opened the company's J.P Morgan Healthcare Conference presentation by reflecting on a transformative decade in gene editing and reaffirming the company's commitment to advancing in vivo CRISPR-based therapeutics. Lenoard emphasized the company's strategic pivot to focus resources on two late-stage assets with blockbuster potential, NTLA-2002 for the treatment of hereditary angioedema (HAE) and Nex-z (formerly NTLA-2001) for the treatment of transthyretin amyloidosis (ATTR).

Intellia is entering a critical phase with three actively enrolling Phase III programs, laying the groundwork for its transition from a research-based biotech to a commercial-stage business. NTLA-2002, a single-dose CRISPR treatment designed to provide a functional cure for HAE, is poised to become the company's first commercial product with results from its Phase III HEALO study and regulatory approval submissions anticipated in 2026. NTLA-2002's blockbuster potential comes from the multibillion-dollar addressable market in HAE and the fact that the drug offers lifelong freedom from attacks and chronic therapy.

Nex-z, targeting both cardiomyopathy and polyneuropathy forms of ATTR, is anticipated to follow with a potential launch in 2030. Although preliminary and not from a head-to-head study, Phase I/II data shows Nex-z has demonstrated superior reductions in serum TTR than the existing therapy Vutrisiran, with results suggesting significant clinical and disease regression.

Leonard emphasized that the company's strategic reprioritization would ensure clinical and commercial readiness, with sufficient cash runway to sustain operations through commercialization. Intellia's operational priorities for 2025 include completing enrollment for the HALO study of NTLA-2002 and the MAGNITUDE and MAGNITUDE-2 studies for Nex-z. As Intellia advances its Phase III programs and prepares for its first regulatory

submissions, the company is firmly positioned to achieve delivering transformative, first-in-class therapies that fundamentally improve patient outcomes. Leonard concluded with confidence in Intellia's trajectory, declaring 2025 as a pivotal year in the company's journey to unlocking the full potential of CRISPR-based gene editing.

Nuvation Bio

Founder and CEO, Dr. David Hung, kicked off the presentation for Nuvation Bio at the 43rd Annual J.P. Morgan Healthcare Conference. Dr. Hung was previously the co-founder and CEO of Medivation (acquired by Pfizer in 2016 for a total enterprise value of approximately $14B), the developers of Xtandi. Nuvation is a biopharmaceutical company developing treatments for difficult-to-treat cancers by addressing drug resistance while maintaining quality of life and reducing side effects. Nuvation's lead program, Taletrectinib, is a 3rd generation, potentially best-in-class ROS1 inhibitor and was approved for advanced ROS1+ NSCLC in China in December 2024. Taletrectinib was granted Orphan Drug designation and Breakthrough Therapy designation in the U.S. An NDA was accepted by the FDA for priority with a PDUFA date assigned for June 23, 2025. Dr. Hung emphasized Nuvation is well positioned to be a U.S. commercial stage organization by mid 2025 in a sizeable commercial opportunity where there are only currently three approved treatments in the U.S. to treat patients with ROS1+ NSCLC. Phase II data from the TRUST-I and TRUST-II studies of taletrectinib for advanced ROS-1 positive NSCLC earlier presented at ASCO and ESMO in 2024 were reiterated in the presentation. The pooled analysis from the TRUST-I and TRUST-II studies offer meaningful advancements in efficacy and a favorable safety profile. In TKI-naïve patients, tumors shrank in 89% of taletrectinib-treated patients (cORR) and the median PFS was 46 months. In TKI-pretreated patients, tumors shrank 56% and median PFS was 10 months.

Nuvation is also developing Phase II safusidenib, a MIDH1 inhibitor, and Phase I NUV-1511, the company's first clinical stage drug-drug conjugate (DDC) for advanced solid tumors and Phase I NUV-868, a BD2-selectivve BET inhibitor for advanced solid tumors. Safusidenib is expected to enter pivotal studies in 2205 for diffuse IDH1-mutant glioma with a Phase II study currently ongoing. Nuvation's confidence to have a successful commercial launch brings upon Xtandi launch experience of the commercial officer and head of sales from Medivation. Nuvation currently is in a strong cash position with $549.1M as of September 30, 2024 for ongoing operations in the near term.

Protagonist Therapeutics

Protagonist Therapeutics (PTGX) president and CEO Dinesh Patel, MD, led the company's JPM presentation which focused on progress and upcoming milestones for Protagonist's

two lead peptide therapeutics, rusfertide and icotrokinra. Being developed in partnership with Takeda, rusfertide is a subcutaneous weekly hepcidin mimetic in Phase III studies for polycythemia vera (PV), a rare myeloproliferative neoplasm characterized by overproduction of red blood cells. The condition affects about 155k patients in the US, the majority of whom have uncontrolled hematocrit on current stand-of-care therapies. With no red blood cell-specific pharmaceutical options available, Protagonist sought to design a hepcidin mimetic that could effectively treat PV. Takeda licensed exclusive rights to co-develop and commercialize rusfertide in January 2024, and the partners have seen positive Phase III results, with 88% of patients in the REVIVE study progressing to the long-term THRIVE study. Protagonist expects top-line results in March 2025, and an NDA filing by year-end. With the candidate's rapid, consistent, and sustained hematocrit control, the companies believe rusfertide has peak revenue potential of $1-2 billion.

Dr. Patel also noted that an NDA filing is expected by year-end for icotrokinra, the oral IL-23 receptor antagonist peptide in development with partner Johnson & Johnson. This candidate has best-in-class potential and is a first-in-class and only-in-class oral treatment for psoriasis, as an alternative to current injectable therapies. Successful Phase III results announced in late 2024 earned Protagonist a $165 million milestone payment from J&J, $115 million of which was for the Phase III first endpoint achievement, and $50 million of which was accelerated payments related to NDA approval and Phase III data on a second indication. In addition to psoriasis, studies are ongoing for ulcerative colitis. Icotrokinra has blockbuster potential as an oral, once-daily medication. Of the patients currently on injectable psoriasis therapies, 75% note that they would switch to an oral if available.

The presentation concluded with discussion on the company's earlier-stage candidates – PN-881, a potential best-in-class oral peptide interleukin-17 (IL-17) antagonist for immune-mediated skin diseases (IND-enabling studies in progress), an oral obesity project, and an oral hepcidin candidate.

With the milestone and accelerated payment received from J&J in November, Dr. Patel notes that the company's cash runway will extend through at least the end of 2028.

Vir Biotechnology

Marianne De Backer, the CEO of Vir Biotechnology, kicked off the presentation with their compelling mission statement: harness the power of the immune system to fight back against cancer and viruses and transform patient's lives. She emphasized that Vir Biotechnology is at a pivotal juncture, with several programs yielding promising data and a well-defined trajectory that could offer new hope and improve outcomes for patients. At the core of Vir's strategy is their PRO-XTEN masked TCE program, which includes

VIR-5818 and VIR-5500, designed to target cancer. De Backer presented a striking case study where PRO-XTEN masked TCEs demonstrated dramatic patient responses in a patient with HER2-positive breast cancer, resulting in a 52% reduction in tumor size from baseline, while maintaining a well-tolerated safety profile. She also highlighted early Phase I data in HER2-positive colorectal cancer (CRC) patients who had exhausted standard therapies. In this cohort, confirmed partial responses were observed in 33% of participants at early doses, with one patient continuing treatment for over 18 months, demonstrating favorable tolerability. Lastly for their PRO-XTENs, De Backer reiterated data from their Phase I study of VIR-5500 in mCRPC, where PSA reduction responses were observed across all 12 patients, with a tolerable safety profile. Furthermore, De Backer underscored Phase I results from VIR-5500 in metastatic castration-resistant prostate cancer (mCRPC), where prostate-specific antigen (PSA) reductions were observed in all 12 patients, coupled with an acceptable safety profile. She also gave an honorable mention to VIR-5525, which holds the potential to unlock multiple high-value indications, including metastatic non-small cell lung cancer (mNSCLC), metastatic colorectal cancer (mCRC), and metastatic head and neck squamous cell carcinoma (mHNSCC). Vir is poised and excited to initiate a Phase I study of VIR-5525 in the first half of this year.

The latter portion of the presentation focused on the significant unmet need for treatments targeting Hepatitis Delta, a disease currently without any approved therapies. De Backer discussed the elevated risk of liver cancer in patients with Hepatitis D and B, highlighting the transformative potential of Vir's treatments in these populations. She presented data from their ongoing Phase II trial for Hepatitis Delta virus (HDV), which combines tobevibart and elebsiran to induce substantial virological responses. These results demonstrated profound antiviral effects, with 41% of patients achieving undetectable HDV virus levels at 24 weeks, and 64% at 36 weeks, with continued deepening of responses over time. Vir is enthusiastic about initiating their Phase III ECLIPSE program for HDV this year, with ECLIPSE 1 and 2 supporting marketing applications in the U.S. and Europe, and ECLIPSE 3 providing additional evidence for value creation outside the U.S., supporting reimbursement, and facilitating label expansion. De Backer also provided a brief overview of the design for these three studies.

As of January, Vir holds approximately $1.1 billion in cash and investments, with sufficient runway to fund operations through mid-2027. The presentation concluded with a discussion of upcoming milestones, including the initiation of the ECLIPSE registrational studies in the first half of 2025, the launch of a Phase I study for VIR-5525, and additional data on hepatitis B expected in Q2 2025. With a robust portfolio in both oncology and infectious disease, Vir Biotechnology is well-positioned for significant near-term value creation.

Zymeworks

At the start of his presentation, Chair and CEO Kenneth Galbraith acknowledged 2024 as a pivotal year for Zymeworks (ZYME). The company has a product pipeline which focuses on building uniquely differentiated agents through antibody engineering. In

November, the FDA granted accelerated approval of the internally developed Ziihera® (zanidatamab-hrii) for the treatment of adults with previously treated, unresectable or metastatic HER2-positive (IHC 3+) biliary tract cancer (BTC). Regulatory decisions in the EU and China are expected this year for zanidatamab in second-line BTC with potential approval as early as 2Q 2025. Partner Jazz Pharmaceuticals is expected to report top-line results from the Phase 3 HERIZON-GEA-01 trial evaluating zanidatamab in HER2-positive GEA in 2Q2025 with potential for submission for a sBLA in first-line GEA later in 2025 (potential launch in 2026).

The momentum from Ziihera® successes is likely to propel the development of Zymeworks' six wholly owned candidates including the 2 clinical stage assets in Phase 1 Trials: ZW171 & ZW191. ZW171 is a 2+1 trivalent T cell engager targeting mesothelin-expressing solid tumors. ZW191 is an antibody-drug conjugate (ADC) engineered to target folate receptor-*a* utilizing a novel proprietary topoisomerase 1 inhibitor (TOPO1i) payload, ZD-06519. Galbraith indicated data for both studies is anticipated to be shared in 2025 through peer-reviewed publications. Zymeworks will continue to drive the progression of its pipeline of ADCs and multispecific antibody therapeutics (MSATs), targeting completion of all five IND applications by the end of 1H2026.

Zymeworks will leverage its proprietary platforms to develop its AD- VAN-CE portfolio beyond solid tumors in indications such as AIID and hematological cancers. The first-in-class multi-functional therapeutics consist of novel payload ADCs (novel payloads and targets), cell engagers (multi-specific T cell engagers, multi-antigen targeting) and cytokine engineering (tumor specific cytokine activation, combination checkpoint inhibition/cytokine activation). Submission of the first IND application in AIID expected in 2H 2026 for ZW1528, with a focus on COPD.

Galbraith emphasized Zymeworks is well positioned to deliver on its mission to improve the standard of care for difficult to treat diseases. The R&D successes achieved over the past year will enable the company to focus on opportunities to advance its diverse portfolio of novel, multifunctional biotherapeutics through internal innovation and strategic collaborations. With the receipt of certain anticipated regulatory milestone payments, Zymeworks anticipates a cash runway into the second half of 2027.

Micro Cap

Akebia Therapeutics

Akebia Therapeutics, a late-stage renal biopharmaceutical company centred around addressing the complications of chronic kidney disease (CKD), presented its prospects for 2025 at the JP Morgan Healthcare conference, underpinned by the recent launch of Vafseo (vadadustat) for the treatment anemia in dialysis-dependent CKD. With near-complete market access in the US secured through contracts covering nearly 100% of all dialysis provided, Vafseo is positioned to disrupt a multibillion market that is currently dominated by injectable erythropoiesis-stimulating agents (ESAs). Vafseo offers a different approach by stimulating the body's natural response to hypoxia, enabling gradual and sustained increases in hemoglobin without the risks associated with ESAs. Akebia sees key opportunities within the high-dose ESA patient segment, which comprises approximately 150,000 in the US, for whom Vafseo offers a safer and more convenient treatment option with less risk of cardiovascular events. Additionally, the growing home dialysis market, which is estimated at 80,000 patients, offers another setting in which Vafseo's oral formulation could thrive.

The dialysis market is only the beginning for Akebia, as the company is aiming to establish Vafseo as the new standard-of-care for all CKD-related anemia, including the dialysis-independent patient segment. Substantial unmet need remains in the treatment of anemia in dialysis-independent CKD patients, where ESAs have been declining in use due to issues with injection regiments and instability in hemoglobin levels. Akebia has been working with the FDA to design a Phase III cardiovascular outcomes study targeting late-stage non-dialysis patients, which will compare Vafseo to the standard-of-care and is expected to start mid-2025.

Beyond Vafseo, Akebia has cultivated its expertise in hypoxia-inducible factor (HIF) biology and plans to bring additional therapies to market. AKB-9090, a novel HIF-prolyl hydroxylase inhibitor HIF-PHI), is planned to enter clinical studies by the end of 2025, and will be studies in both cardiac surgery-associated acute kidney injury and acute respiratory distress syndrome. Marking Akebia's first movement outside of kidney disease, AKB-10108, another HIF-PF inhibitor, is being developed for the rare disease, retinopathy of prematurity, which has no approved treatment and a $1.5 billion US market opportunity. With a robust pipeline, a solid financial foundation, and a strong initial reception for Vafseo, Akebia is well-positioned for growth in the coming years. The company is focused on executing its dialysis launch, driving demand, and continuing to

build clinical evidence, all while preparing to expand Vafseo's reach into the non-dialysis CKD market. As 2025 progresses, Akebia is poised to continue its mission to transform kidney disease treatment and improve the lives of millions of patients.

Amneal Pharmaceuticals

Amneal Pharmaceuticals' Co-Chief Executive Officer and President Chirag Patel started the presentation with an overview of the company's growth over the past five years. Amneal launches 20-30 quality drugs per year and has grown 11% over 5 years. The company currently has a diverse portfolio of over 280 generic and specialty pharmaceuticals, primarily within the United States, with a focus on generics and complex innovations in biosimilars and injectables. Patel outlined plans to expand by growing the CREXONT® business following its launch late last year, and investing in opportunities in specialty assets, biosimilars and the emerging global GLP-1 space. CREXONT® has shown strong initial adoption with ~1% market share in 3 months since launch for Parkinson's Disease (PD) in September 2024. The current payor coverage is at ~30% and expected to be at 50%+ by end of 2025, well ahead of the RYTARY® trend. Amneal is moving forward with its specialty business primarily in neurology and endocrinology. Following 2024's successful launches in PD (ONGENTYS® in Q1 and CREXONT® in September), Patel indicated the potential first and only DHE prefilled syringe autoinjector for the acute treatment of migraine and cluster headaches in adult will be available in Q2. Patel also noted Amneal's intention to expand on its position in the biosimilars space, citing the attractiveness of affordable medicines, the mounting opportunities due to branded products losing exclusivity (including key biologics) and the limited number of players. It is expected that Amneal will have 6 commercial biosimilars across 8 therapies by 2027 (6 oncology, 1 bone health, 1 allergy). The company intends to expand its biosimilar portfolio through in-licensing (1-2 biosimilars/year) and vertically integrating across development, manufacturing and commercial operations. In closing, Patel highlighted the growth projections across all business areas. Amneal is well-positioned to continue driving sustainable top and bottom-line growth each year with ~15% revenue growth and ~11% adjusted EBITDA growth in 2024.

Annexon Biosciences

Annexon Biosciences' (ANNX) President and CEO Douglas Love opened the presentation with a broad overview of the company's progress in becoming a leader in complement disease landscape. With a validated scientific platform revolving around the company's founding discovery C1q, a complement component beginning the classical pathway of innate immune response, Annexon promises a broad potential pipeline across multiple therapeutic areas, spearheaded by the lead program ANX005 to treat Guillain-Barré Syndrome (GBS).

Annexon's ANX005 is a single-dose therapy intended to inhibit C1q and the entire classical complement pathway to treat GBS. GBS is a severe disease resulting from an acute autoimmune attack on peripheral nerves following activation of C1q and the classical complement cascade. Currently, no treatments are approved for GBS by the FDA. Most GBS patients are treated with off-label intravenous immunoglobulin (IVIg) in the US, but it carries numerous disadvantages including lengthy treatment period (5 days of daily infusions) and a non-specific treatment approach. As such, Mr. Love stressed that given the urgent nature of GBS requiring rapid drug onset, ANX005 could effectively address this unmet need as a single-dose monotherapy. In the Phase III study, ANX005 demonstrated rapid increase in muscle strength and translated these improvements into long term recovery. Since the Phase III study was conducted in Southeast Asia, a real-world evidence study was required to establish compatibility between the Phase III population and Western GBS patients from IGOS database. The real-world analysis noted fewer patients treated with ANX005 required mechanical ventilation assistance and fewer days in the ICU compared to standard of care. Altogether, Mr. Love highlighted the blockbuster potential for ANX005 as the first targeted therapy for GBS to replace the standard of care. A Biologics Licensing Application (BLA) submission is expected in the first half of 2025.

Mr. Love shifted focus to ANX007, a novel therapy for the treatment of Geographic Atrophy (GA). Treatment with ANX007 in a Phase II study showed preservation of visual acuity and protection of structures associated with visual function, the first demonstration by a complement-based therapy. Building on the results from the Phase II ARCHER study, the Phase III ARCHER II trial aims to evaluate monthly ANX007 against placebo injection in GA patients. The primary endpoint is persistent BCVA ≥15-letter loss through 12 months. The study is projected to complete enrollment in the second half of 2025, with data readout expected in 2026.

Rounding out the pipeline discussion is ANX1502, an oral small molecule complement therapy. Given many treatments for complement-mediated diseases are biologics and carry inconvenience injection administration burden for both physicians and patients, ANX1502 seeks to address this inflexibility via its oral dosing administration. In addition, as an oral molecule targeting the active form of C1s, ANX1502 could target multiple indications via inhibition of classical complement pathway. Given encouraging proof-of-concept data from a Phase I program, Annexon plans to advance ANX1502 through clinical stages with more updates on future complement-mediated indications in early 2025.

Closing out the presentation, Mr. Love reiterated a strong momentum for Annexon to be continued through 2025 with a potential BLA submission for ANX005 in GBS in the first half of 2025, Phase III enrollment completion for ANX007 in GA in the second half of 2025, and clinical updates for ANX1502, the first oral small molecule complement therapy. Lastly, the company has enough cash runway through the second half of 2026,

ensuring sufficient capital to fund clinical and commercial operations to launch ANX005 if approved.

AngioDynamics

Jim Clemmer, President and Chief Executive Officer for AngioDynamics presented an overview of the company's recent history, its novel technology and plans for the future. Built on a legacy of established medical devices, AngioDynamics is developing new medical technologies to treat cardiovascular disease and cancer. The new platforms currently target venous thromboembolism (VTE), peripheral arterial disease (PAD) and prostate cancer, but Clemmer noted their potential for wider use. Since 2020, the company has streamlined its portfolio of products and the recent focus on medical technology has resulted in an increase in revenue from this segment of the business increasing from approximately 15% to approximately 45%.

Prostate cancer is increasing in prevalence with aging populations, but treatment options are suboptimal. Clemmer highlighted the current challenges men with prostate cancer face with management options including observation at one end of the spectrum and radical prostatectomy and radiation therapy at the other. These latter interventions are effective but can lead to erectile dysfunction (ED) and urinary incontinence (UI). Consequently, there is a need for focal therapy that can target just the affected areas. AngioDynamics' NanoKnife involves the placement of probes in the target tissue and the administration of electrical pulses that cause cellular death within specific margins reducing the risk of complications. Data from the pivotal PRESERVE trial showed that, after 12 months, 84% of NanoKnife recipients had no infield disease and less than 10% were affected by ED and only 1.2% developed UI, much lower complication rates than the conventional treatment approaches.

AngioDynamics' cardiovascular disease focuses are VTE (pulmonary embolism [PE] and deep vein thrombosis [DVT]) and PAD. Mechanical thrombectomy is increasingly being used to treat VTE but the market is currently under penetrated according to Clemmer. AngioDynamics' AngioVac system is an established thrombectomy product that allows for continuous clot aspiration with the effective vortex funnel tip and reinfusion of blood minimizing volume loss. The company's new AlphaVac system combines the AngioVac funnel tip with a large bore cannula and off-circuit manual aspiration control providing a tool with greater aspiration and maneuverability. Data from the APEX pivotal trial in PE patients showed that AlphaVac achieved approximately three-fold clot reduction versus competitors. For PAD, the company launched the Auryon atherectomy system in 2020 and it is now achieving sales in excess of $150 million, taking market share from major competitors. Auryon uses a laser to generate shockwaves to break up arterial calcifications and it is now being investigated for use in coronary arterial disease. Funding for research and development has been possible due to the company's medical device portfolio which provides solid revenue stream and has helped the company to

become debt free. AngioDynamics' financial year runs from June to May with year to date sales of more than $140 million and full year sales expected to approach $300 million. Moreover, the company is already generating profit with a positive outlook for growth based on the high margin medical technology segment of the business. Stephen Trowbridge, Executive Vice President and Chief Financial Officer, joined Clemer following the presentation and they highlighted how the company has strengthened despite the challenges of the covid pandemic for the business and it is looking to grow further by moving manufacturing offshore and expanding the geographic footprint for its leading products.

Anika Therapeutics

At the 43rd Annual J.P. Morgan Healthcare Conference, Anika Therapeutics shared updates on its financial performance, product pipeline, and strategic goals. The company is in a solid financial position, with $62 million in cash and no debt, and an expected 2024 revenue of $117 to $121 million. Growth in its commercial channel is projected to increase by 14% to 19% year-over-year. The company continues to see steady contributions from its well-established osteoarthritis (OA) pain management portfolio, which includes Monovisc and Orthovisc, both of which maintain strong market positions in the US.

Anika highlighted key developments in its product pipeline, including the Integrity Implant System, which has grown over 40% sequentially since its U.S. launch in late 2023, targeting the $220 million tendon augmentation market. Hyalofast, an innovative regenerative scaffold for cartilage repair, is moving toward a U.S. launch in 2026, with the potential to address a $1 billion market. Meanwhile, Cingal, a next-generation OA pain product with strong clinical data showing significant and durable pain relief, is expected to be a major growth driver following its U.S. launch, pending FDA approval. Looking ahead, Anika is focused on scaling the Integrity Implant System, preparing for the mid-term launch of Hyalofast, and paving the way for the longer-term rollout of Cingal. Together, these initiatives target a total addressable market exceeding $3 billion. By combining financial discipline with a clear focus on these high-impact programs, Anika aims to expand its leadership in regenerative solutions and OA pain management, leveraging its innovative hyaluronic acid platform to meet growing patient and market needs.

Arcturus Therapeutics

Joseph E Payne, President and Chief Executive Officer of Arcturus Therapeutics provided an overview of the technology supporting product development, the status of the pipeline and current development plans. The company has a broad intellectual property portfolio based on messenger RNA (mRNA) technology, such as the self-amplifying

mRNA (STARR®) low-dose vaccine technology and the LUNAR® delivery system, a proprietary, biodegradable lipid nanoparticle technology optimized for different cell types, including hepatocytes (intravenous administration), myocytes (intramuscular), and bronchial cells (inhaled). Compared with conventional mRNA vaccines, STARR® provide a superior, broader and more durable immune responses, at a lower dose level and with more rapid manufacturing. LUNAR® utilizes an endosome to deliver mRNA to targeted cells.

Arcturus has partnered with CSL for the development three Kostaive covid-19 vaccine candidates (monovalent [ARCT-154], bivalent [ARCT-2301], and a candidate for the XBB.1.5 variant [ARCT-2303],) as well as a seasonal influenza candidate. CSL reports $14.8 billion in annual revenue and operations in more than 40 countries. It has 13 Phase III programs based on four strategic technology platforms (plasma protein; recombinant technology; cell and gene therapy; and vaccines) to provide products across immunology, hematology, respiratory, cardiovascular, transplant, nephrology and vaccines. The global partnership with CSL could realize up to $4.3 billion in milestone payments for Arcturus. The Meiji Group will be involved with the Japanese development and commercialization of the Kostaive covid-19 vaccines. Working with Meiji and Axcelead, Arcturus have also helped establish Arcalis, a contract development and manufacturing organization specializing in the manufacture of mRNA vaccines and therapeutics. Results from the Phase III trials of monovalent and bivalent Kostaive vaccines versus Comirnaty (Pfizer and BioNTech's COVID-19 mRNA vaccine) led to approval in Japan, a world's first for a self-amplifying mRNA product, plus a positive opinion from the EMA's Committee for Medicinal Products for Human Use (CHMP); filing with the US FDA is also planned. The company is also working with the US Biomedical Advanced Research and Development Authority for development of a pandemic H5N1 influenza vaccine candidate owing to the increased risk posed by this strain to people and animal industries; a Phase I trial is underway.

Payne then provided more details on the mRNA therapeutics Arcturus have in development. LUNAR®-CF (ARCT-032) is an inhaled mRNA therapeutic candidate for cystic fibrosis (CF), with funding of $25 million from the CF Foundation. CF affects up to 100,000 people and is caused by mutations in the CFTR gene, resulting in poor chloride transport and dehydrated, sticky mucus in the airways leading to chronic airway obstruction, infection and inflammation, which damages the airway and eventually leads to respiratory failure requiring lung transplant. There are CFTR modulators approved but many patients are not able to receive these treatments, and they cannot prevent the progressive loss of lung function. LUNAR®-CF could restore CFTR function through mRNA replacement producing wild-type CFTR that will create a functional protein restoring chloride efflux in the airways. Preclinical studies in animal models have shown that LUNAR-CF can reach the bronchial cells and restore mucociliary clearance, with CFTR expression and function also restored in experiments involving huma bronchial cells. Phase I trials have been positive and Phase II data from CF patients is expected in

H1 2025. This drug candidate has received Rare Pediatric Disease Designation and Orphan Drug Designation from the US FDA and Orphan Medicinal Product Designation from the European Commission (EC). LUNAR®-OTC (ARCT-810) is systemically delivered mRNA for ornithine transcarbamylase (OTC) deficiency, a rare liver disease (affecting more than 10,000 people) that is the most common urea cycle disorder. This disease results in elevated blood ammonia, which can lead to neurological damage and death. The current standard of care includes ammonia scavengers, but these agents are unable to prevent life-threatening spikes of ammonia, meaning that liver transplant is the only cure. LUNAR®-OTC aims to restart enzyme function that will express OTC and restore urea cycle activity. Animal models have suggested efficacy that exceeds that minimum 5% activity required to avoid the worst symptoms and outcomes. Posive Phase I data have led to a Phase 2 trial for which interim results are expected in H1 2025. This drug candidate has received Rare Pediatric Disease Designation, Fast Track and Orphan Drug Designation from the FDA and the EC has awarded it Orphan Medicinal Product Designation.

Payne concluded by highlighting the prospects of revenues from the successful commercialization of the company's first product in Japan potentially followed by European and US approval and the anticipation of the first Phase II data from their other novel therapeutic candidates in the first half of 2025.

Caribou Biosciences

Caribou Biosciences is a CRISPR genome editing company that is using its CRISPR hybrid RNA-DNA (chRDNA) platform to develop a wholly owned pipeline of off-the-shelf CAR-T cell therapies for a variety of heme malignancies and autoimmune diseases. Caribou has three allogeneic CAR-T cell therapies in the clinic: CB-010 targeting CD19 for DLBCL and lupus, CB-011 targeting BCMA for multiple myeloma and CB-012 targeting CLL-1 for AML. The advantage of allogeneic CAR-T cells over currently approved autologous CAR-T cells include access (more patients can be treated with an off-the-shelf therapy compared to a therapy that has to be custom manufactured), speed (in clinical trials, patients are starting lymphodepletion two days after eligibility versus weeks to months for autologous CAR-Ts) and scale (>300 doses/manufacturing run versus 1 dose/run for autologous CAR-T).

CB-010 is the most advanced asset and has three edits: introduction of the anti-CD19 CAR and knockout of PD-1 (to reduce CAR-T cell exhaustion) and the T scell receptor (to avoid graft-versus-host disease). ANTLER is a Phase I trial evaluating a single dose of CB-010. The dose expansion part of the trial dosed 30 2L DLBCL patients and results were presented at ASCO 2024. Interestingly, a post-hoc analysis found that the most durable responses were found in 10 patients with at least 4 HLA matches to the CAR-T donor (out of 12 loci). These 10 patients also showed improvement in pharmacokinetic

measures such as peak expansion and persistence. The trial is now enrolling a cohort of 20 additional patients with this HLA matching strategy. A second cohort is enrolling 10 patients who relapsed with prior CD19 treatment. Data from these cohorts are expected in H1 2025 and if they are positive, a pivotal Phase III trial will be initiated in H2 2025. Caribou CEO Rachel Haurwitz noted that currently only 20% of 2L DLBCL patients are being treated with CAR-T and that approval of an allogeneic CAR-T has the potential to increase this percentage.

Apart from DLBCL, CB-010 is also in development for the larger indication of lupus. An off-the-shelf CAR-T option will be important for meeting the needs of such a large indication. Furthermore, there is no need for apheresis for allogeneic CAR-Ts thereby avoiding the need for washing out lupus drugs prior to apheresis. The Phase I GALLOP trial is evaluating a single dose of CD-010 at the recommended Phase II dose determined from the ANTLER trial and is enrolling two cohorts in parallel, one for patients with lupus nephritis and one for patients with extrarenal lupus. Caribou will provide updates as the trial advances.

CB-011 is armored with four edits to reduce rejection and improve antitumor activity. These edits include removing β2-microglubulin (B2M) and inserting a B2M-HLA-E fusion protein. B2M stabilizes all HLA class I antigens on the cell surface, and so its knockout eliminates endogenous HLA class I presentation on the surface of the CAR-T cells thereby reducing T cell mediated rejection. HLA-E is a minor HLA antigen but its expression through the B2M-HLA-E fusion protein prevents rejection by NK cells. The other two edits are the TCR knockout and the introduction of the anti-BCMA CAR. The Phase I CaMMouflage trial is enrolling 4L+ multiple myeloma patients but excludes patients with prior CAR-T cell therapy and/or BCMA-targeted therapy within the last three months. The trial evaluated a lymphodepleting regimen of 30/300 fludarabine/cyclophosphamide and doses of 50M, 150M and 450M cells but efficacy was improved with deeper lymphodepletion (30/500 fludarabine/cyclophosphamide). Dose escalation is continuing at 450-800m cells with the deeper lymphodepletion. The dose escalation data (minimum of 15 patients at active doses) will be presented in H1 2025. The final CAR-T in a clinical trial is CB-012 which has the potential to be the first CAR-T cell therapy approved for relapsed/refractory AML. CB-012 has five edits: knockout of the TCR, B2M and PD-1 and introduction of the anti-CLL-1 CAR and the B2M-HLA-E fusion protein. The AMpLify Phase I trial is enrolling 2L-4L patients and is in dose-escalation. Caribou will provide updates as the trial advances. Caribou ended Q3 2024 with $281m cash on hand providing it with a runway into H2 2026.

C4 Therapeutics

C4 Therapeutics is a clinical stage company that is developing a portfolio of catalytic, orally bioavailable, targeted protein degraders. Cemsidomide is the most advanced

protein degrader and is being developed for multiple myeloma and PTCL. It targets the transcription factor IKZF1/3 involved in differentiation of hematopoietic stem cells into myeloid and lymphoid progenitor cells. Degrading IKZF1/3 leads to downregulation of IRF4 promoting myeloma and lymphoma cell death and the on-target side effect of neutropenia. IKFZ1/3 degradation can also lead to some activation of T cells which may make it a suitable partner for T cell engaging bispecific antibodies. Cemsidomide is differentiated from other degraders such as the approved compounds lenalidomide and pomalidomide and the developmental stage compounds iberdomide and mezigdomide by its best in-class potency and by its increased selectivity for IKZF1/3 resulting in reduced off-target toxicity. Results from a Phase I trial were presented at ASH 2024 and showed encouraging results for cemsidomide + dexamethasone (dex) in heavily pretreated multiple myeloma patients. A Phase II trial designed to support an accelerated approval will evaluate this combination in late-line patients previously treated with an anti-BCMA therapy and is expected to initiate in early 2026. C4 Therapeutics is also planning two pivotal Phase III trials. The first trial will evaluate an anti-CD38 antibody + cemsidomide + dex in 3L-5L patients previously treated with an anti-BCMA antibody while the second trial will evaluate an anti-BCMA bispecific antibody + cemsidomide in 2L-4L patients. In the latter trial, cemsidomide has the potential to enhance response durability and treatment duration of the BCMA bispecific by preventing T cell exhaustion.

Cemsidomide monotherapy has also shown activity in a Phase I trial in patients with NHL. The NHL data support further development in PTCL which provides the fastest path to market. A Phase II trial with the potential to support accelerated approval for 2L+ relapsed/refractory PTCL is expected to initiate in 2026. C4 Therapeutics is also planning a pivotal Phase III trial in the 1L PTCL setting that will evaluate cemisdomide combined with standard of care therapy (Adcetris ± chemotherapy for CD30+ disease or CHOP chemotherapy for CD30- disease).

The second degrader in clinical development is CFT1946 which targets the V600 BRAF mutant. While BRAF inhibitors have been approved, resistance mechanisms lead to limited duration of response. Furthermore, skin toxicities associated with inhibition of wild-type BRAF limit tolerability. CFT1946 has been developed to avoid resistance mechanisms and to spare wild-type BRAF. In addition, CFT1946, can cross the blood brain barrier highlighting its potential for drug delivery to CNS metastases. Encouraging data from a Phase I/II dose escalation trial were presented at ESMO 2024 showing activity of CFT1946 monotherapy in patients who progressed after treatment with BRAF inhibitors. Further Phase I data are expected in 2025 including cohorts evaluating monotherapy or a combination with Mekinist for melanoma, an indication where the BRAF V600 mutation rate is 35%, and a cohort evaluating a combination with Erbitux for colorectal cancer, an indication where the BRAF V600 mutation rate is 5-10%. Data from these Phase I cohorts will help determine the next phase of development which for melanoma could include CFT1946 ± a MEK inhibitor for BRAF inhibitor exposed patients, CFT1946 + a MEK inhibitor for BRAF inhibitor naïve patients and CFT1946 + immune

checkpoint inhibitor for front-line patients. For colorectal cancer, CFT1946 could be combined with Erbitux for frontline patients.

The final degrader is CFT8919 which targets L858R EGFR for NSCLC and which initiated its first clinical trial in China in late 2024 led by partner Betta Pharmaceuticals. With current EGFR inhibitors, patients may become refractory due to secondary mutations. CFT8919 exploits an allosteric binding site created by the L858R mutation, thereby avoiding resistance mutations to the orthosteric site. Preclinical work has shown that CFT8919 is potent and selective against L858R regardless of secondary mutations with potential for more durable activity in this setting. CFT8919 has also been shown to not degrade wild-type EGFR potentially resulting in better tolerability than currently approved EGFR inhibitors. Data from the Phase I trial will be used to determine whether to initiate development in the US which could include treatment for patients that relapse with secondary EGFR mutations such as C797S. Positive data for these three assets will help C4 Therapeutics become a fully integrated biotech company focusing on oral bioavailable degraders.

Immunome

At the J.P. Morgan Healthcare Conference, Immunome's CEO Dr. Clay Siegall outlined recent business developments and anticipated milestones for 2025.
Immunome's long-term corporate vision is to develop differentiated ADCs with a focus on first-in-class targets. The company plans to achieve this goal through numerous efforts including internal discovery efforts with dozens of novel targets under evaluation and pipeline expansion driven by M&A. Per the CEO, the company is currently well positioned for corporate partnerships and maintains a cash runway that is expected to extend into 2026.

Immunome discussed a few anticipated catalysts for 2025 in the presentation. Topline Phase III data from the Phase II/III RINGSIDE trial of varegacestat (formerly AL102) are expected in the second half of 2025. Varegacestat is being evaluated as an oral, once-daily gamma secretase inhibitor for the treatment of desmoid tumors. Previous data from Phase II of the study showed increased response compared to nirogacestat across all measures. If approved, varegacestat represents an opportunity to establish a new standard of care.

In the earlier stage of the company's pipeline, IM-1021 is expected to enter Phase I evaluation in solid tumors and B-cell lymphoma in the first quarter of 2025. IM-1021 is a ROR1 ADC with an HC74 payload selected for properties that enable efficacy in both solid and liquid tumors. Immunome believes that the compound has significant commercial opportunities and the potential for accelerated approval in the United States. Additionally, an IND submission for IM-3050, a 177Lu-FAP radiotherapy, is expected in

the first quarter of 2025. The company also has three novel ADCs against solid tumor targets, IM-1617, IM-1340, and IM-1335, that are currently in IND enabling studies. Six additional ADCs are undergoing lead optimization with development decisions expected in 2025/2026.

Lexeo Therapeutics

By leveraging advancements in gene therapy and cardiac-targeting technologies to address significant unmet needs in cardiac diseases, Lexeo Therapeutics is positioning itself as a leader in the field of cardiac genetic medicine. The company focuses on developing gene therapies for conditions with limited or no existing disease-modifying therapies. Financially, Lexeo concluded 2024 with approximately $157 million in cash and marketable securities, providing a projected cash runway into 2027. In this presentation, CEO Nolan Townsend focused on Lexeo's lead assets: LX2006, which targets Friedreich Ataxia (FA) cardiomyopathy, and LX2020, for Plakophilin 2 (PKP2) arrhythmogenic cardiomyopathy (ACM).

FA is a rare, devastating and progressive disorder caused by loss of function mutations in the FXN gene. The only approved disease-specific treatment for FA demonstrated efficacy on neurological measures but it was not evaluated for the treatment of cardiac function. LX2006 is the only program in the clinic for the treatment of FA cardiopathy, which is the cause of death in 60-80% of FA patients. The gene therapy has the potential to treat the root cause of FA cardiomyopathy (the significant decrease in frataxin in the heart) and is Lexeo's most advanced program in development, with 16 participants dosed thus far. Lexeo announced interim data from the Phase I/II SUNRISE-FA trial in July 2024, demonstrating promising results, with reductions in left ventricular mass index (LVMI) and increases in frataxin protein expression, both key markers of clinical improvement in FA cardiomyopathy. Literature shows a 19% incremental risk of all-cause mortality per ~10% increase in LVMI. Earlier in 2024, Lexeo entered a licensing agreement with Cornell University, securing intellectual property related to LX2006, further enhancing its strategic position.

PKP2-ACM is a rare, genetic, cardiac disorder caused by loss of function mutations in the PKP2 gene. Current management methods are focused on relieving symptoms and preventing sudden cardiac death (which approximately 23% of patients experience) and do not address the underlying cause of myocardial dysfunction and ACM. LX2020 is being evaluated in the ongoing Phase I/II study, HEROIC-PKP2, with Cohort 1 fully enrolled. Preclinical data demonstrated improvement across key areas for determining ACM diagnosis and risk profile, such as arrhythmia burden, depolarization and repolarization abnormalities, and cardiac contractibility. There are approximately 60,000 patients in the US who could benefit from LX2020.

Looking ahead, we expect an update regarding the LX2006 program in mid-2025, including biopsies from high-dose Cohort 3, and longer duration cardiac biomarker data across all cohorts. Lexeo's recent alignment with the FDA guides future pivotal study inclusion criteria and co-primary endpoints to support the accelerated approval of LX2006. A registrational study, which has the potential to be initiated at the end of 2025 or early 2026, will enroll patients with abnormal LVMI at baseline. The co-primary endpoints will be based around a reduction in LVMI (a threshold of 10% reduction) and an increase in frataxin expression (a threshold of 40% frataxin positive area). Regarding LX020, initial clinical data, focused on protein expression and safety, are expected from Cohort 1 of the HEROIC-PKP2 study in late Q1 or early Q2, 2025. Lexeo plans to give an update on the LX2020 program in the second half of 2025, including data from Cohort 1 and 2.

The company is also pursuing partnership opportunities for the continued development of another of its assets, LX1001, which is a gene therapy in development for APOE4-associated Alzheimer's disease (AD). Interim Phase I/II data were released for LX1001 in October 2024, with treatment leading to dose-dependent increases in APOE2 protein expression and improvements in AD-associated tau biomarkers, but the sample size was small, and larger trials are needed to confirm LX1001's efficacy and safety.

Neumora

Neumora Therapeutics is a neuroscience-focused biotechnology company aiming to redefine treatment paradigms for some of the most common and impactful psychiatric and neurodegenerative diseases. The company has three clinical-stage assets in development for major depressive disorder (MDD), bipolar depression, schizophrenia, and agitation in Alzheimer's disease, as well as a preclinical program exploring mechanisms in amyotrophic lateral sclerosis (ALS) and Parkinson's disease, as well as the latter two aforementioned indications. With $850M raised since 2021, Neumora has a cash runway extending to mid-2026, supporting its robust growth plans.

The drug that is furthest along is navacaprant, a kappa opioid receptor antagonist in Phase III trials for MDD. Despite showing moderately promising efficacy in Phase II, topline results from the first pivotal Phase III trial, KOASTAL-1, were quite the opposite. Although the safety profile was encouraging across both trials, the drug failed both the primary and key secondary endpoints in Phase III. This was a surprising result that the company is attributing to a high placebo response, particularly in male participants, and an abnormal distribution of male versus female subjects. Neumora's financial stability has allowed researchers to deeply scrutinize the KOASTAL-1 data with the intention of implementing the findings into the ongoing staggered KOASTAL-2 and KOASTAL-3 trials and hopefully mitigating the potential for more failed pivotal trials.

Aside from the registrational program for navacaprant in MDD, Neumora also has two signal-seeking studies underway: navacaprant in bipolar II and NMRA-511, a vasopressin 1a receptor antagonist, in Alzheimer's disease agitation. Data for the latter are expected in the second half of 2025. Though NMRA-266, an early-stage M4 positive allosteric modulator for schizophrenia, remains on clinical hold, the company plans to submit an investigational new drug (IND) application for one of its other M4-targeting agents in the first half of the year. With the advancing of preclinical assets and the implementation of lessons learned from KOASTAL-1, 2025 is poised to be a year of growth for Neumora.

Precigen

Precigen's (PGEN) opening remarks highlighted the foundation and potential of its precision medicine platform AdenoVerse. According to the Chief Executive Officer (CEO) Helen Sabzevari, the AdenoVerse platform promises great potential because it offers a large payload capacity, durable immune response, and a unique ability for repeat administration with higher efficiency in manufacturing.
The majority of the presentation centered on the development and commercial readiness of PRGN-2012, Precigen's flagship program. PRGN-2012 (zopapogene imadenovec) is an AdenoVerse gene therapy designed to elicit immune responses directed against cells infected with human papillomavirus (HPV) 6 or HPV 11 for the treatment of recurrent respiratory papillomatosis (RRP). RRP is a HPV-driven rare disease with no FDA-approved therapeutic. In RRP patients, benign papillomas grow in the respiratory tract and can cause severe voice disturbance and potentially obstruct airway. The current standard of care is repeat surgical debulking of papilloma, which can lead to irreversible scarring of the trachea. If approved, PRGN-2012 could address this significant unmet need for a disease-modifying therapy to avoid surgical complications.

In 2024, Precigen submitted a Biologics License Application (BLA) for PRGN-2012 based on the positive results from the pivotal Phase I/II study. In the Phase I/II study, 51% of patients demonstrated complete response, defined as no RRP surgeries in 12 months following treatment. Moreover, 86% of subjects demonstrated a reduction in the need for surgeries after treatment. Importantly, these responses are durable; the median duration of response has not been reached after a median follow-up of 20 months. On safety, PRGN-2012 treatment was well-tolerated with no dose limiting toxicities, no treatment discontinuations, and no meaningful anti-drug antibodies response. Given the recently completed BLA submission, an approval decision is expected in 2025, on track for a potential launch in the second half of 2025.

Interestingly, Precigen noted that the increase in Gardasil vaccination, a HPV vaccine, is unlikely to affect the prevalence of RRP despite being a HPV-driven disease. The company projected an estimated 70% of patients are expected to remain unvaccinated by 2040. An internal market analysis of RRP disease market revealed approximately

27,000 prevalent cases in the US and over 125,000 prevalent cases outside of the US, confirming the significant unmet need. As such, Precigen suggests that PRGN-2012 has blockbuster potential in RRP given its first-to-market advantage and the likelihood of becoming the standard of care for first line RRP.

Beyond PRGN-2012, the presentation discussed the latest R&D progress for UltraCAR-T platform. According to the company, the decentralized overnight manufacturing process and rapid gene transfer protocol could significantly reduce the wait time for autologous CAR-T treatment. Subsequently, it would streamline implementation at many medical centers and allow next-day infusion for patients. Leading the UltraCAR-T platform is PRGN-3006, an autologous CAR-T cell therapy for acute myeloid leukemia (AML). A Phase I trial recently completed enrollment, and preliminary analysis of patient samples has identified clinical biomarkers that correlate to objective responses after PRGN-3006 treatment. More clinical development is expected for this asset, beginning with the engagement with the US FDA to discuss these findings and the next steps. Sabzevari also discussed the potential of partnerships to advance this program.

Looking ahead, Precigen continues to realize its commercial vision for PRGN-2012. As the company ramps up its launch preparation with a fully functional in-house cGMP facility in Germantown, MD, Precigen is poised to introduce the first RRP approved therapy in 2025, should it be approved. In terms of the company's overall cash balance, Sabzevari noted an unaudited $100 million in cash with runway into 2026, well past the anticipated launch of PRGN-2012. Beyond the market readiness activities, the company is planning to maximize the value of its UltraCAR-T platform through clinical development and strategic partnerships to advance PRGN-3006 program in AML. With the launch of PRGN-2012 and ongoing work on the UltraCAR-T platform, Precigen is well positioned for significant near-term value creation.

ProKidney

ProKidney is an autologous cell therapy company focused on achieving preservation of kidney function in patients with type 2 diabetes and advanced chronic kidney disease (CKD), a population that is at very high risk of progressing to end-stage renal disease. The company's flagship asset, Rilparencel or ReACT (renal autologous cell therapy), involves harvesting renal tissue with a typical kidney biopsy, which then undergoes subsequent expansion and selection, before being re-injected back into the patients' damaged kidneys. Despite significant treatment advances, including the approvals of several SGLT-2 inhibitors for the treatment of CKD, a significant number of patients continue to lose kidney function and progress to advanced CKD. As such, there is still a substantial unmet need in CKD and Rilparencel is designed to address this gap by stabilizing and preserving renal function.

CEO Bruce Culleton outlined the company's progress over the last year, including a successful meeting with the FDA regarding the possibility of accelerated approval for Rilparencel using estimated glomerular filtration rate (eGFR) slope, as well as the relaunch of the pivotal Phase III PROACT-1 trial. This 600-patient randomized study in individuals with diabetes and CKD is utilizing a composite endpoint of renal and cardiovascular outcomes, similarly to many other landmark trials, and results from the study are expected in 2027. Other highlights of 2024 include top-line data from the Phase II REGEN-0007 trial was published in June 2024 and showed Rilparencel stabilized kidney function for 18 months, with an average eGFR change from baseline to 18 months of -1.3 ml/min/1.73m2. Although data are limited, Rilparencel exhibited favorable tolerability with no treatment-related serious adverse events in the 49 participants that received at least one Rilparencel injection. ProKidney also underwent a strategic shift in 2024, which included focusing on bilateral dosing of Rilparencel for greater efficacy as well as discontinuing the ex-US PROACT-2 trial following validation from the FDA that a single confirmatory study would be enough for approval under the Regenerative Medicine Advance Therapy designation that was issued in 2022.

With robust Phase II data, a strong safety and tolerability profile, and a clear regulatory path ahead, ProKidney is poised to redefine CKD treatment by delivering a potentially life-changing therapy to a critically underserved population.

Protara Therapeutics

Protara Therapeutics, a company focused on developing and commercializing rare disease therapies, has presented forward-looking statements regarding its business strategy and active pipeline. The presentation primarily focused on TARA-002, an investigational, genetically distinct strain of Streptococcus pyogenes, an immunopotentiator with significant potential. The asset activates the Th1 immune cascade and has a broad immunopotentiation mechanism similar to BCG but with higher efficacy in early-stage trials. TARA-002's originator therapy, OK-432, has already been approved for lymphatic malformations (LMs) and several oncology indications in Japan since the 1990s. To date, TARA-002 is being investigated in two Phase II trials, the ADVANCED-2 trial in non-muscle-invasive bladder cancer (NMIBC) and STARBORN-1 in LMs, with topline results expected in H2 2025.

The company expects TARA-002 to be widely used in the NMIBC population if late-stage trials prove successful due to its high response rates in both BCG-UN and BCG-naïve patients (80% reinduction salvage rate), an acceptable safety and tolerability profile and lack of contraindications with current treatments. These are particularly significant findings because BCG failure rates are expected to be between 40 and 50% of the patient population in a group with an annual incidence of more than 65,000 in the United States alone. BCG failure is currently treated by an extremely invasive cystectomy. In

this sense, TARA-002 would meet a significant unmet need for both patients and physicians to avoid this treatment. Similarly, TARA-002 is being studied in LMs, a condition with surgical intervention as a treatment option but has substantial complications and recurrence rates, with approximately 1,400-1,800 cases per year. Protara is also investigating phospholipid substrate replacement therapy for patients dependent on parenteral support (PS) to meet nutritional needs. A common complication of PS is the limited absorption of choline increasing the risk of liver disease. Choline deficiency in PS is among the largest rare disease indications, with approximately 30,000 PS patients in the US alone. A Phase II study confirmed choline replacement improved and restored serum choline concentrations and a pronounced impact on steatosis. Choline replacement has already been actively included in guidelines and recommended by key PS professional associations. A pivotal Phase IIb/III THRIVE-3 trial is anticipated to initiate in H1 2025. Protara remains well funded with $81.5m of cash, cash equivalents, and investments as of September 30, 2024. The company anticipates a cash runway into 2027.

RAPT Therapeutics

Presenting on behalf of RAPT Therapeutics at this year's JPM conference was president and CEO Brian Wong, M.D., Ph.D. RAPT's mission is to discover, develop and commercialize transformative therapies for high value inflammatory and immunological diseases. The company leverages its proprietary discovery and development platform to advance both biologics and selective small molecules aimed at normalizing critical immune drivers underlying these conditions.

Lead program RPT904 is a half-life extended omalizumab (Xolair) "bio-better" antibody with potential to transform the treatment of food allergy (FA) and chronic spontaneous urticaria (CSU). It was licensed from Jemincare, a leading pharmaceutical company in China. Phase 1 data supports RPT904 with a potential best-in-class profile, less frequent dosing, and greater patient compliance. RAPT plans to initiate a Phase 2b FA trial in 2H 2025 with topline data expected 1H 2027. It also plans to initial a Phase 2 or Phase 3 trial in CSU in 2026. RAPT projects over $5.5 billion US peak revenue for FA and CSU combined. Jemincare is currently conducting Phase 2 trials of RPT904 in China for asthma and CSU. Topline data for both studies are in expected 2H 2025. Jemincare's Phase 1 trial of RPT904 showed longer half-life and superior IgE reduction compared to omalizumab.

Omalizumab is an emerging blockbuster in the large and growing food allergy market. There are over $17 million FA patients in the US, of which 50% have experienced a severe reaction leading to over three million ER visits per year. Omalizumab is the only FDA-approved therapy to reduce allergic reactions for multiple foods based on the Phase 3 OUtMATCH study. The CSU indication offers an additional commercial upside as it

affects more than 1 million patients in the US. Antihistamines are the first treatment step but approximately 400 thousand patients are not controlled on antihistamines. Omalizumab is the only approved biologic for CSU after the failure of antihistamines. RPT904 is positioned to be the preferred choice in the front-line setting due to improved compliance and convenience compared to omalizumab. Even with efficacy 20% below omalizumab, prescribers still prefer the less frequent dosing for RPT904. RAPT estimates over $1 billion in peak US revenues for the CSU indication. RAPT also has a next-generation oral CCR4 antagonist in discovery with potential to fill high unmet need for a safe oral option for a range of Th2-driven disorders and has improved potency and liver safety margins. Preclinical candidate selection is expected in H1 2025 and clinical trials are anticipated to begin in 2026. Mr. Wong concluded his presentation stating that the company is well funded with cash runway projected through multiple clinical milestones including Phase 2b FA data which is expected in the first half of 2027.

Rigel Pharmaceuticals

Rigel Pharmaceuticals, a hematology and oncology-focused company, experienced a transformational year in 2024, marked by substantial commercial growth and strategic advancements in its development pipeline. These achievements have positioned the company for continued success and future growth, as highlighted in presentations by CEO Raul Rodriguez at a J.P. Morgan conference 2025. Rigel's approach to building a successful biopharmaceutical company is multifaceted, involving strong commercial execution of its current product portfolio, strategic in-licensing and acquisitions, financial discipline, and advancement of key development programs.

The commercial business of Rigel is anchored by three key products: Tavalisse, Rezlidhia, and Gavreto, which have collectively driven significant revenue growth. Tavalisse (fostamatinib) is indicated for the treatment of adult chronic immune thrombocytopenia (ITP), a condition where the body destroys its own platelets, leading to a risk of severe bleeding. In Q4 2024, Tavalisse achieved $31 million in net product sales, a 16% growth compared to Q4 2023. This growth demonstrates the product's strong market presence and continued adoption by clinicians. Rezlidhia (olutasidenib), indicated for adult patients with acute or refractory AML with a susceptible IDH1 mutation, has also shown impressive growth since its launch. In Q4 2024, Rezlidhia reached $7.4 million in net product sales, an impressive 92% increase over the previous quarter last year. The company has identified a strong potential for this product beyond AML in other hematologic conditions and gliomas. Gavreto (pralsetinib) is used to treat RET fusion-positive non-small cell lung cancer and thyroid cancer. Rigel acquired US rights to Gavreto in 2024 from Blueprint Medicines. In its first full quarter (Q3 2024) under Rigel, Gavreto generated $7.1 million in sales, which increased to $8.1 million in Q4 2024, demonstrating the company's ability to manage product transfers and expand its market presence effectively. Overall, Rigel's net product sales reached $145 million in

2024, representing a 38% increase from the previous year, with a 58% growth in Q4 over the same quarter of the last year. This performance underscores the company's strong revenue generation capabilities.

In addition to the organic growth of its product portfolio, Rigel has strategically expanded its offerings through in-licensing and product acquisition, as seen with the addition of Rezlidhia and Gavreto. Rigel is also investing in advancing its development programs in significant unmet medical need areas. A key focus is R289, a dual IRAK 1 and 4 inhibitor, which was discovered by Rigel and is currently in a Phase 1b study for lower-risk myelodysplastic syndromes (MDS). R289 has received Fast Track and Orphan Drug designations. The Phase 1b study evaluates the safety, tolerability, and preliminary activity of R289 in patients with relapsed or refractory lower-risk MDS. Early data has been encouraging, with approximately 40% of evaluable patients achieving a response with R289 doses ≥ 500mg QD. The company expects to complete the dose escalation portion of the study in 2025 and initiate the expansion phase. Rigel is also looking at expanding the use of olutasidenib beyond AML and into glioma. Gliomas are common brain tumors with a high unmet need, especially in cases of relapse. A Phase 1b/2 trial has shown preliminary clinical activity. In 2025 Rigel is planning a Phase 2 clinical study in recurrent glioma, in collaboration with CONNECT, and a study combining olutasidenib with other agents at MD Anderson Cancer Center. Rigel partnered with Lilly to develop RIPK1 inhibitors for immune and CNS diseases.

Rigel's focus on commercial success and strategic development positions the company to drive future growth. The company has issued guidance for 2025, anticipating total revenue of $200 million to $210 million and positive net income while continuing to fund clinical development programs.

Sage

Sage Therapeutics is a biopharmaceutical company dedicated to pioneering treatments for neurological and psychiatric disorders. The core priorities for the upcoming year are expanding the commercialization of Zurzuvae and enhancing financial focus through pipeline prioritization. However, just days before the JPM conference, Biogen, Sage’s US partner in the development and commercialization of Zurzuvae, made an unsolicited buyout offer valuing the company at $470 million. While public comments were understandably restricted, Sage indicated that the board plans to fulfill its fiduciary responsibility and discuss Biogen’s offer.

Sage’s portfolio is relatively limited, particularly following the streamlining efforts last year. Zurzuvae, a neurosteroid for postpartum depression (PPD), is the sole marketed asset after the 2024 discontinuation of its flagship offering, Zulresso. Like Zulresso, Zurzuvae is a short-course, positive allosteric modulator (PAM) at the GABAA receptor, but has a more convenient oral formulation compared to the former’s intravenous

administration. The majority of Sage's presentation was focused on the ongoing commercialization efforts, which lean on OB-GYN education as well as social media influencer campaigns to help combat the stigma surrounding PPD. This approach has been successful thus far, with an impressive 90% brand awareness one year after launch and consistent increases in quarter-over-quarter prescriptions. Coverage of the drug has also improved, both for Medicaid and commercially insured patients, and the guidelines for treating PPD from the American College of Obstetricians and Gynecologists specifically mention Zurzuvae as an option. Moreover, Shionogi, Sage's Zurzuvae partner in Japan, Taiwan and South Korea, has submitted an application for Zurzuvae in major depressive disorder (MDD) in Japan. MDD was originally part of Sage's US strategy as well, but the FDA rejected this indication in 2023 and opted to approve the drug only for PPD.

The recalibrated pipeline is comprised of a few early-stage assets, with a main focus on SAGE-319 and SAGE-324. The former is an extra-synaptic GABA PAM currently being assessed in a Phase I multiple ascending dose study. Data from this trial are expected in late 2025 and Sage hopes to move into patient studies for behavioral symptoms in neurodevelopmental disorders, such as autism spectrum disorder, in 2026. SAGE-324, a drug with a similar mechanism to SAGE-319, is still under evaluation, potentially for seizures in developmental and epileptic encephalopathies, and an update on next steps will be shared in mid-2025. Should the company decide to pursue development, this would expand their primary offerings outside of behavioral indications. As it stands, due to 2024 restructuring efforts, the company has a cash runway through mid-2027 to fund the ambitious plans for Zurzuvae and the development of select pipeline candidates.

Seres Therapeutics

Seres Therapeutics is a company which aims to transform outcomes through the development of live biotherapeutics. The presentation was led by Chief Executive Officer Eric Shaff who began by emphasizing how strong the financial position of the company is following the completed sale of their product Vowst, which occurred in September 2024. Vowst was the first FDA-approved, orally administered, microbiome therapeutic which was developed to prevent the occurrence of Clostridium difficile infection in adults. A payment of $50 million is expected to be received during January 2025, with a further $25 million expected in July 2025. There is also the potential for future sales-based milestone payments to total $275 million.

The main priorities of the company are to continue developing the current pipeline of novel live biotherapeutics in disease areas which have high unmet needs. Seres' pipeline is based on the idea that a disrupted gastrointestinal microbiome is linked to the development of infections and disease and therefore has substantial untapped therapeutic potential. For example, bloodstream infections (BSIs) are a leading cause of

mortality following transplant and incidence is increasing. The company will begin by further advancing their most advanced asset SER-155 following positive Phase Ib trial results in allogeneic hematopoietic stem cell transplantation (allo-HSCT).

This acceleration will also be bolstered by the FDA Breakthrough Therapy designation that SER-155 received in December 2024. In addition, Seres also wants to pursue a strategic partnership to accelerate the next stage of SER-155 clinical development and also allow for an expansion into multiple target populations. Seres is confident that SER-155 has the commercial potential to become a blockbuster drug in the treatment of allo-HSCT. This is a disease area that has high unmet need as approximately 40,000 patients worldwide receive a transplant annually, and there is a requirement to prevent the subsequent development of serious infections. The company also has plans to advance its early-stage pipeline candidate SER-147 for preventing infections in chronic liver disease, following positive preclinical data.

Shattuck Labs

Shattuck Labs, Inc. (STTK) CEO Taylor Schreiber, MD, PhD, opened the presentation highlighting development of the company's DR3 (death receptor 3) antibody blocking program and the clinically validated rationale for targeting the TL1A (tumor necrosis factor (TNF)-like ligand 1A) and DR3, the receptor for TL1A.

Dr. Schreiber cited Phase II data presented last year on the TL1A-blocking antibody tulisokibart, a compound originated by Prometheus (acquired by Merck in 2023), that showed the monotherapy delivered a 25% placebo-adjusted completed remission rate in patients with treatment-refractory ulcerative colitis (UC). This data was also replicated across other Phase II/III clinical trials in antibodies under development by both Roche and Teva/Sanofi, showing across all three studies clinical remission rates of 20-27%, thus providing strong validation for Shattuck's targeting of this pathway.

Efforts to date have focused on blocking just the ligand, TL1A, which is expressed only transiently in tissue at sites of inflammation, whereas the DR3 receptor is expressed constitutively on circulating and tissue-resident lymphocytes in both involved and uninvolved areas of the gut. Shattuck is the only company to date developing a DR3-targeted antibody. Because DR3 targeting can more broadly suppress TL1A signaling across the GI tract of UC and Crohn's disease (CD) patients, the company is hoping its SL-325 program, a potential first-in-class DR3 blocking antibody, as a monotherapy, can close the gap between the approximately two-thirds of patients that have some degree of clinical response from a TL1A inhibitor and the approximate 25% that are able to achieve clinical remission. Dr. Schrieber gave a detailed explanation of the biology and expression of TL1A and DR3 in IBD patients and referenced multiple studies showing DR3 inhibition is potentially more potent than TL1A inhibition. RNA sequencing data for

UC and CD patient biopsies confirmed constitutive and higher expression of DR3 than TL1A in the GI tract, as well as in the peripheral blood, where TL1A is not.

Also presented was an overview of Shattuck's lead candidate SL-325, which has demonstrated the ability to block TL1A binding to DR3 in vitro at an approximately 10-fold greater potency than benchmark anti-TL1A antibodies. It was shown to block TL1A induced IFN gamma cytokine secretion from IBD patient peripheral blood cells and efficiently blocked IFN gamma across all tested IBD patient samples, showing potential for higher rates of endoscopic remission, with no risk of soluble TL1A mAb immune complex formation and no interference with prior TL1A treatment. Regarding the rest of the pipeline, SL-425, an equivalent monoclonal antibody to SL-325, with an extended half-life, was mentioned, as well as bispecifics in early development that leverage the DR3 moiety. Also highlighted was the opportunity for development of other antibodies using this approach.

Expected milestones for SL-325 in the near term include the presentation of the preclinical data readout from the recently completed GLP toxicology study in nonhuman primates at the European Crohn's and Colitis Congress meeting in February. SL-325's entrance to the clinic is anticipated in the middle of this year, with first in human single- and multi-ascending dose clinical trials expected to be fully completed by Q2 2026, and then followed by a randomized, placebo-controlled study in patients with CD and a separate study in UC. The company's cash and cash equivalents are projected to fund operations and planned development into 2027.

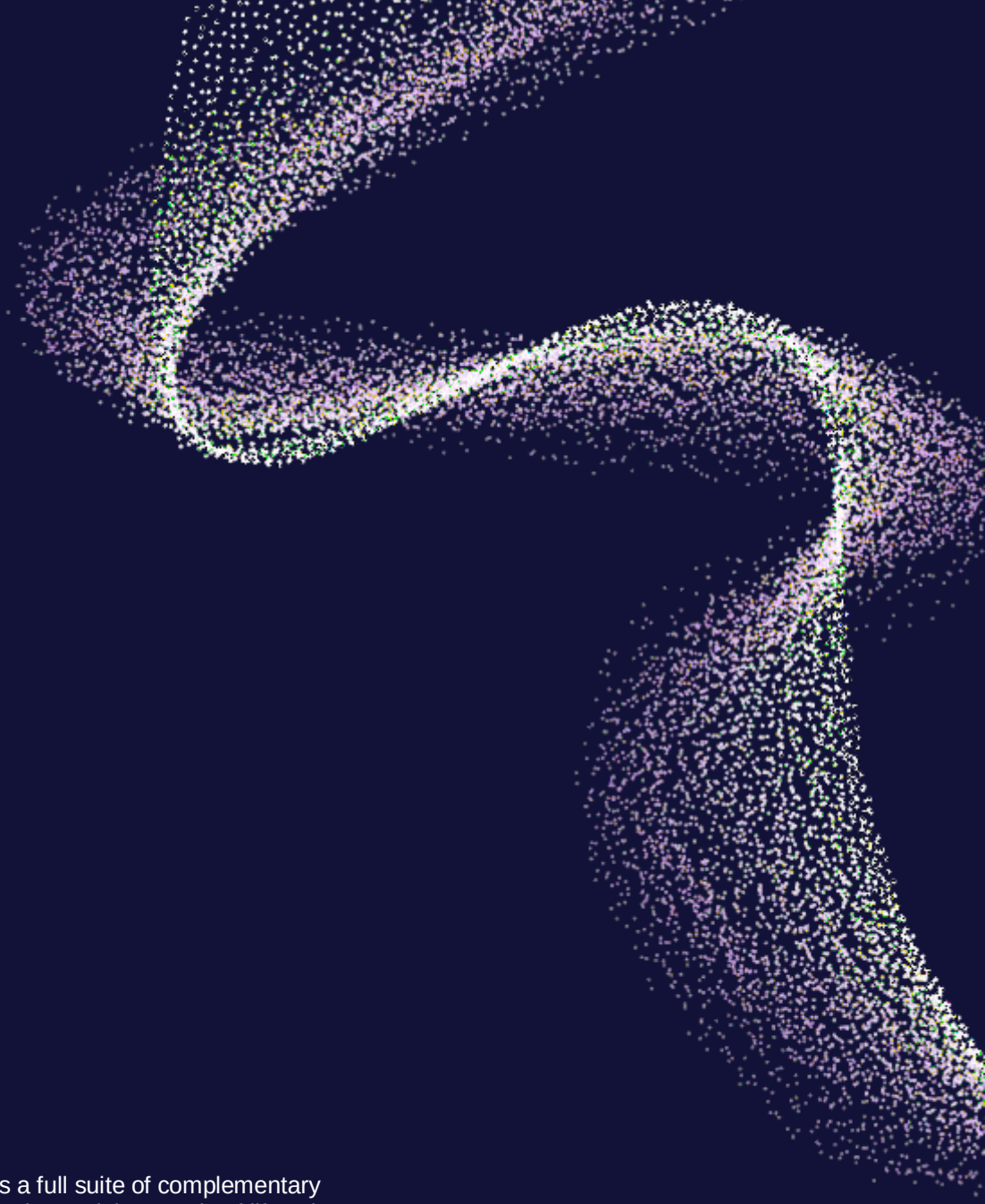
Skye Bioscience

Founded in 2011, Skye Bioscience is a biopharmaceutical company dedicated to developing treatments for metabolic health, including obesity, through molecules modulating G-protein coupled receptors to improve health and quality of life for patients. At the 43rd Annual J.P. Morgan Healthcare Conference, Skye Bioscience started off by reviewing its missions and selecting financials. Since August 2023, $107M in equity capital has been raised backed by specialist life science investors and collaborators.

Since then, Skye was uplisted to Nasdaq in April 2024 and added to the Russell 2000 in July 2024. Skye is funded through the third quarter of 2027 with continued strategic investments in manufacturing, operations, and R&D to advance its pipeline.
Skye's lead program, nimacimab, is a first-in-class Cannabinoid Receptor 1 (CB1)-inhibiting antibody. By targeting the CB1 receptor in peripheral tissues in the liver, adipose, and muscle allows nimacimab to cause fat breakdown with better gastrointestinal tolerability and muscle preservation to result in sustainable weight loss. Nimacimab has demonstrated a tolerable safety profile and is currently being studied in the Phase II Cbeyond trial, initiated in August 2024, with the primary endpoint of

percent change in body weight at baseline, week 26 and week 38 as well as safety and tolerability and neuropsychiatric and change in body composition and waist circumferences as its secondary endpoints. Skye anticipates releasing interim and topline results from Cbeyond throughout 2025. A Phase IIb trial is planned for 2026 as well as an End-of-Phase II meeting with the FDA and Phase III planning in 2027.

CITELINE
COMMERCIAL



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

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