BofA SECURITIES

Biotechnology

Catalyst expectations for 2024

Price Objective Change

SRPT: Elevidys label expansion expected by August

Sarepta (SRPT, Buy, $164 PO) continues with the launch of Elevidys following the initial approval in 4–5-year-old Duchenne muscular dystrophy (DMD) patients. Despite an initial restricted label, the Elevidys launch has exceeding Street expectations reporting ~$200mn in the first 6 months. The company is now focused on securing a label expansion to expand the addressable patient population. The efficacy supplement requesting the expansion of the Elevidys label to all DMD patients regardless of age or ambulatory status and conversion to traditional approval from accelerated approval was submitted in December (see our Dec 22 note). Management noted that under normal regulatory timelines they expect acceptance of the filing in early March and an action date in August. However, they noted they anticipate a more expedited review is possible given the large unmet need. We remain confident that a label expansion is likely, given commentary from management and recent remarks from the FDA's Peter Marks on the agency's commitment to facilitating the approval of more gene therapies for rare diseases (see our Jan 9 note). Other 2024 priorities include, continuing to expand the rest of the pipeline with plans to report data for SRP-5051 (next generation exon skipping therapy) and focusing on advancing the ongoing pivotal trial for SRP-9003 in limb-girdle muscular dystrophy type 2E. We continue to be encouraged by the early metrics of the Elevidys launch and we view a label expansion to all DMD patients as likely based on encouraging feedback from regulators. We maintain our Buy with $164 PO.

See inside for detailed catalyst on Acadia (ACAD), Alnylam (ALNY), Annexon (ANNX), Apellis (APLS), Argenx (ARGX), Ascendis (ASND), BioCryst (BCRX), BioNTech (BNTX), Denali (DNLI), Amicus (FOLD), Incyte (INCY), Neurocrine (NBIX), Pharvaris (PHVS), PTC (PTCT), Sage (SAGE) and Ultragenyx (RARE) and Sage (SAGE; PO change from $26 to $30).

17 January 2024

Equity
United States
Biotechnology

Tazeen Ahmad
Research Analyst
BofAS
+1 646 855 4236
tazeen.ahmad@bofa.com

Daniel Giraldo
Research Analyst
BofAS
daniel.giraldoperez@bofa.com

Jeremiah Lorentz
Research Analyst
BofAS
jeremiah.lorentz@bofa.com

Exhibit 1: PO changes in this report
Summary of PO changes

Ticker	Previous	Current
SAGE	$26	$30

Source: BofA Global Research

BofA GLOBAL RESEARCH

Abbreviations

DMD: Duchenne muscular dystrophy

FDA: Food and Drug administration

LGDM: Limb-girdle muscular dystrophy

Refer to important disclosures on page 18 to 21. Analyst Certification on page 17. Price Objective Basis/Risk on page 12.

ACAD: Topline ph 3 data in NSOS in 1Q24

Acadia Pharma (ACAD, Neutral, $33 PO) remains focused on the Daybue launch in Rett syndrome (RS), reporting strong 3Q sales of $66.9mn for the therapy's first full quarter on the market. Management has provided 4Q23 sales guidance of $80-87.5mn for Daybue, however we model 4Q revenue of $93mn based on positive feedback from Key opinion leaders (KOLs) on current usage trends (see our Daybue usage survey). We highlight our continued focus on updated launch metrics at future earnings calls for insight on the real-world retention rate, which will help provide color on long-term Daybue opportunity. We currently model $997mn in risk-adjusted peak sales for Daybue. On Nuplazid expansion opportunity, ACAD expects to release topline data for the phase 3 ADVANCE-2 study evaluating pimavanserin in negative symptoms of schizophrenia (NSOS) in 1Q24. If positive, management anticipates submitting a supplemental new drug application (sNDA) later in the year. Ahead of the readout, we plan to do more work surveying our KOLs to help set expectations and to determine what level of data would be needed for a US approval. We currently do not breakout standalone value for the program and include it in our $1.4bn pipeline plug given the NSOS space has proven difficult to enter with multiple program failures in recent years. Additional near-term catalysts include: 1) New Drug Submission (NDS) filing for Daybue in Canada (~600-900 RS patients) in 1Q24, 2) engaging with Japanese (~1K-2K RS patients) regulators on a filing path in 2024, 3) developing potential new therapies for Prader-Willi syndrome and Alzheimer's disease psychosis in 2024, and 4) filing a marketing authorization application (MAA) in Europe (~9K-14K RS patients) in 1H25. We maintain our Neutral with $33 PO.

ALNY: KARDIA-2 and -3 readouts in 2024

Alnylam Pharmaceuticals (ALNY, Buy, $246 PO) is expanding its commercial and clinical RNA interference pipeline with near-term focus on vutrisiran in ATTR amyloidosis with cardiomyopathy (ATTR-CM) and zilebesiran in hypertension. After receiving a complete response letter (CRL) for the supplemental new drug application (sNDA) of patisiran in ATTR-CM (see our Oct 9 note), management remains confident in vutrisiran in the same indication. We remain focused on the HELIOS-B topline data in early-24, as we see vutri' in ATTR-CM being the biggest value driver for the company, modeling $3.9bn in risk-adjusted peak sales in ATTR-CM.

ALNY is also planning to report phase 2 KARDIA-2 trial of zilebesiran in hypertension in early-24, likely March/April based on management commentary. KARDIA-2 is evaluating zilebesiran in combination with a single agent. Following the announcement of the deal with Roche (see our July 24 note), ALNY also plans to initiate the phase 2 KARDIA-3 trial in 2024. Our KOLs have highlighted they would see a >5mmHg reduction in systolic blood pressure compared to placebo as clinically meaningful. We expect greater focus on the KARDIA-2 and KARDIA-3 trials given zilebesiran will most likely be used in combination with other agents. We view hypertension as a big market opportunity and await further data to validate the potential for zilebesiran in this large indication. We maintain our Buy with $246 PO.

ANNX: Several programs moving forward in 2024

Annexon Biosciences (ANNX, Buy, $6 PO) expects to report topline phase 3 pivotal data for ANX005 in Guillain-Barre syndrome (GBS) in 2Q24. We think this could be an attractive commercial opportunity for the company, given the significant disease burden of GBS (~12k+ diagnosed yearly in US/UK; 2bn+ in annual hospital spending) and the fact that there are no approved therapies. The company reported phase 1 data for ANX1502, an oral C1q inhibitor, supporting advancement into a proof-of-concept study in Cold Agglutin Disease (CAD) patients, expected to initiate in 1H24 with data in 2H24. We maintain that ANX1502 could be an interesting opportunity for the company based on its oral delivery and will continue looking to management for On ANX007 in geographic atrophy (GA), management has outlined a global pivotal program comprised of two phase 3 trials, ARCHER II (sham-controlled) and ARROW (head-to-head against

Syfovre), expected to initiate in mid- and late-24, respectively. We are encouraged by the potential of ANX007 in GA and the commercial opportunity of ANX005 in GBS and await further data updates from the respective pivotal programs. We maintain our Buy with $6 PO.

APLS: Focus on EU regulatory review outcome

Apellis (APLS, Buy, $77 PO) remains focused on continuing a strong launch trajectory for SYFOVRE in the US after pre-announcing 4Q Syfovre sales of $114mn beating expectations. Near-term, we are focused on the outcome of the EU regulatory review process. Recall, the company announced that they expect the CHMP (committee for medicinal products for human use) to adopt a negative opinion at the Jan 22-25 meeting (see our Dec 14 note). APLS plans to appeal the decision and receive a final recommendation sometime shortly after the April 22-25 meeting and a final European Commission decision in 3Q24. Decisions on Syfovre marketing in geographic atrophy (GA) by local regulatory authorities in other countries are also expected in 1H24. Additionally, topline data from the phase 3 VALIANT study of pegcetacoplan in Immune Complex Membranoproliferative Glomerulonephritis (IC-MPGN) and the phase 2 study of pegcetacoplan in hematopoietic stem cell transplantation-associated thrombotic microangiopathy (HSCT-TMA) are expected in 3Q24 and 2024, respectively. We are encouraged by signs of continued strong demand for Syfovre in the US as physicians have become comfortable with the risk/benefit profile. We continue to look for color on competitive dynamics in GA and regulatory decisions ex-US, but highlight that the US continues to be main driver of our Syfovre estimates. We maintain our Buy with $77 PO.

ARGX: Vyvgart franchise will continue to expand in 2024

Argenx (ARGX, Buy, $557 PO) continues the launch of Vyvgart (efgartigimod) and the subcutaneous (SC) formulation, Vyvgart Hytrulo, for the treatment of generalized myasthenia gravis (gMG). ARGX reported preliminary FY23 Vyvgart revenues of $1.2bn (ours: $1.2bn), with management highlighting a continued focus on geographic expansion of the franchise throughout 2024. The company noted that the supplemental Biologics license application (sBLA) for Vyvgart Hytrulo in Chronic inflammatory demyelinating polyneuropathy (CIDP) has been submitted with a priority review voucher, and a potential launch is anticipated in mid-24. Regulatory submissions for Vyvgart Hytrulo in CIDP in Japan, Europe, China and Canada are expected by year-end. Beyond the commercial performance of Vyvgart, ARGX's near-term focus is on the 5 additional proof-of-concept with topline data reporting in 2024, including: 1) phase 2 RHO evaluating efgartigimod in Sjogren's in 1H24, 2) phase 2 ALPHA evaluating efgar' in post-COVID postural orthostatic tachycardia syndrome (PC-POTS) in 1H24, and 3) phase 2/3 ALKIVIA evaluating efgar' in 3 myositis subsets in 2H24. We remain encouraged by the strong cadence of the launch and several near-term opportunities to expand the commercial opportunity driven by expansion into new indications and geographies. We maintain our Buy with $557 PO.

Additional near-term catalysts include: 1) Japanese regulatory decision on efgar' in Immune thrombocytopenia (ITP) in 1Q24, 2) Japanese and Chinese regulatory decisions on Vyvgart SC in gMG by 1Q24 and year-end, respectively, 3) Switzerland, Australia, Saudi Arabia and South Korea regulatory decisions on efgar' in gMG by year-end, 4) initiation of seronegative trial of efgar' in gMG by year-end, 5) updates on the development of pre-filled syringes (VYVGART SC in MG and CIDP) in 1H24, 6) initiation of phase 1b/2a studies of ARGX-119 in congenital myasthenic syndrome and ALS in 2024, and 7) 4 new candidates (ARGX-213 (FcRN), ARGX-121 and ARGX-220 (new targets), and ARGX-109 (IL-6)) with INDs expected by YE25.

ASND: Focus on TransCon PTH May 14th PDUFA date

Ascendis Pharma (ASND, Buy, $145 PO) is focused on bringing their second therapy to market with the May 14th prescription drug user fee act (PDUFA) date for TransCon PTH. We note that management highlighted that commercial launch prep has already been completed in the US and it will be ready to launch quickly if granted approval. On

Yorvipath (TransCon PTH) in Germany, the company expects to launch the therapy in January 2024 using its existing Skytrofa salesforce. Management estimates that roughly 22k adult chronic hypoparathyroidism (HPT) patients will be eligible for therapy. On Skytrofa, we continue to be impressed by the strong launch and model €325mn in FY24 sales in line with company guidance of €320-340mn. In future earnings calls, we look for color on the current Skytrofa GHD market share as well as expectations for 2024 as daily injectables continue to exit the market. We maintain our Buy rating with $145 PO given the continued strong Skytrofa launch and note high focus on the TransCon PTH May 14th PDUFA.

Other additional near-term catalysts include: 1) sBLA (supplementary Biologics License Application) submission for Skytrofa in adult growth hormone deficient (GHD) patients in 2Q24, 2) topline results for the phase 3 ApproaCH trial evaluating TransCon CNP in achondroplasia in 4Q24. Management guides to a quick turnaround expecting to submit an NDA (New Drug Application) for pediatric achondroplasia patients also in 4Q24, 3) IND application to evaluate TransCon CNP in adult achondroplasia patients in 4Q24, 4) COACH trial (TransCon hGH / TransCon CNP combination) topline results in 4Q24, 5) topline results for phase 3 trial evaluating TransCon hGH in Turner syndrome in 4Q24, 6) complete enrollment for phase 2 BelieveIT-202 trial (advanced head and neck squamous cell carcinoma) in 4Q24, 7) provide a clinical update on the phase 2 IL-Believe trial (solid tumors) in 4Q24, and 8) creating a spinout ophthalmology (Ophthalmology NewCo) focused company in 1Q24.

BCRX: Mgmt targets $800mn in Orladeyo sales by 2029

Biocryst (BCRX, Buy, $11 PO) provided FY24 Orladeyo revenue guidance of between $380-$400mn (ours: $390mn), and continues efforts to expand access for increased Orladeyo use for prophylactic treatment of hereditary angioedema (HAE). Specifically, we highlight the company's ongoing efforts to reduce the number of patients on free drug. Currently, ~27% of patients are on free drug, but management has the long-term goal of getting 85% of patients on paid drug given the current reimbursement dynamics for new patients. We look for additional color on reimbursement trends in future earnings calls as well as metrics regarding their growing market share despite the crowded HAE market. BCRX targets $800mn in revenue by 2029 highlighting several key drivers: 1) add ~200 net patients each year, 2) improve rate of patients on paid drug, 3) several modest price increases, 4) 15-20% gross-to-net, and 5) low-90s% compliance. While we are encouraged by the company's long-term revenue guidance, we think the expectation for linear growth trajectory still needs validation from future sales trends especially given Orladeyo could see increased competition from newer therapies entering the space. We currently model $692.2mn in peak Orladeyo sales in 2029. We maintain our Buy rating with $11 PO given our view that the current market price is an attractive entry position.

Other additional near-term catalysts include: 1) proof-of-concept (POC) data for BCX10013, an oral Factor D inhibitor, in 2024. Management guides to either partnering or terminating the program depending on the POC data, 2) initiating phase 1 trial for BCX17725 in Netherton Syndrome in 2024, 3) conducting formulation and nonclinical work for avoralstat in 2024, 4) completing lead optimization for an oral C5 inhibitor in 2024, and 6) selecting the lead bifunctional complement inhibitor molecule in 2024.

BNTX: Vaccine and oncology franchises look to expand

BioNTech (BNTX, Buy, $159 PO) provided FY24 COVID revenue guidance of €3bn (ours: €2.5bn). The company expects the transition to a private market in the US with a higher gross price ($110-130/dose). We continue to look for clarity on COVID revenue dynamics for the rest of the year that could give more color on similarities between the COVID and the flu market, and mid-term demand outlook for the COVID vaccine. The company highlighted that they plan to continue shifting to commercialization model in major markets in 2024 and also reiterated their focus on expanding the vaccine business to COVID combination vaccines with other respiratory viruses. We also note that the

company maintains their goal of building a global commercial infrastructure in oncology to support multiple oncology launches starting in 2026, and we await upcoming updates in 2024 as several programs move into registrational trials. We maintain our Buy with $159 PO.

Additional 2024 data catalysts include: 1) phase 2 data for BNT311 (anti-PD-L1/4-1BB bispecific) +/- pembrolizumab in relapsed refractory metastatic non-small cell lung cancer, 2) phase 1/2 expansion cohort data for BNT312 (anti-CD40/4-1BB bispecific), BNT316 (anti-CTLA-4) and BNT323 (anti-HER2) in multiple solid tumors, 3) phase 1/2 data for BNT325 (anti-TROP2) in multiple solid tumors, 4) phase 2 data for BNT327 (anti-PD-L1/VEGF bispecific) in multiple solid tumors, 5) phase 2/3 data for Omicron XBB.1.5 monovalent vaccine, and 6) an update on the phase 1 trial of BNT167 (Shingles vaccine).

DNLI: Phase 2 topline data in ALS expected in 1H

Denali Therapeutics (DNLI, Buy, $29 PO) continues advancing its transport vehicle (TV) and small molecule platform targeting several neurodegenerative diseases. On DNL310 in Hunter syndrome, the company highlighted they anticipate to complete enrollment of the phase 2/3 COMPASS trial in 2024, and expect to provide updates on the phase 1/2 study at WORLDSymposium (Feb 4-9) and Society for the Study of Inborn Errors of Metabolism (SSIEM) (Sep 3-6), which will include longer follow-up safety and biomarker data. Management commented that while they continue to engage with regulators on the potential for accelerated approval based on heparan sulfate reduction, their base case is to file after the full COMPASS readout. The company also announced they expect topline phase 2 data from HIMALAYA (led by partner Sanofi) evaluating SAR443820/DNL788 in amyotrophic lateral sclerosis (ALS) in 1H24 and plan to initiate a phase 1/2 trial in Sanfilippo syndrome type A in early-24 and present initial biomarker and safety data by YE24. We are encouraged by the breadth of DNLI's platform and await further updates from the pipeline to further validate the clinical programs. We maintain our Buy with $29 PO.

Additional near-term catalysts include: 1) presenting preclinical data for DNL126 at WORLD (Feb 4-9), 2) biomarker proof-of-concept and safety data from phase 1/2 study of DNL126 in Sanfilippo syndrome in late-24, 3) IND-enabling studies in the next wave of TV-enabled assets, OTV-MAPT in Alzheimer's disease and OTV-SNCA in Parkinson's disease in 2024, 4) completing enrollment of the phase 2/3 HEALEY study evaluating DNL343 in ALS in 2024. DNLI also plans to continue several ongoing trials in 2024, including Part B of phase 1/2 study of DNL593 in frontotemporal dementia caused by GRN gene mutations (FTD-GRN) in 2024. On partner-led programs, the company will continue and phase 2 studies in Ulcerative colitis and Multiple sclerosis with Sanofi, and a phase 2 study in Parkinson's disease with Biogen.

FOLD: Continuing to look for color on P&O launch metrics

Amicus Therapeutics (FOLD, Buy, $19 PO) remains focused on the launch of Pombiliti & Opfolda (P&O) in the U.S., EU, and U.K. for the treatment of Pompe Disease (PD). Amicus preannounced FY23 P&O revenues of ~$11.6mn (ours: $10.1mn, cons: $10mn). On Galafold in Fabry disease, the company preannounced FY23 revenues of ~387.8mn (+21% y/y, ours: $3852mn, cons: $386mn) and guided to 11-16% growth at constant exchange rate for FY24. We now model FY24 Galafold revenues of $439mn (+13% y/y) in-line with guidance and FY24 P&O revenues of $112.8mn. The company notes that the launch of P&O is going well in all 3 major late onset Pompe disease (LOPD) markets (Germany, UK and US) and the conversion of patients is moving ahead of schedule (see our Nov 8 note). FOLD expects to continue geographic expansion with multiple regulatory submissions in 2024. We are encouraged by continued signs of growth for Galafold and will look for additional launch metrics for P&O in Pompe. We maintain our Buy rating with $19 PO.

INCY: Guidance needed for commercial outlook in '24

Incyte (INCY, Neutral, $69 PO) continues with the launch of its first dermatology product, Opzelura, in atopic dermatitis (AD) and vitiligo. We remain focused on the cadence of the launch, which has so far progressed well, as well as gross-to-net (GtN) dynamics. GtN in 3Q23 was 54% down from 55% in 2Q, with the company commenting they expect GtN to continue around this level and that any improvement would depend on the evolution of Medicaid utilization. Management noted the potential to expand the commercial opportunity of Opzelura into pediatric atopic dermatitis (2-3mn patients) with a supplemental biologics license application expected by mid-24. On Jakafi (ruxolitinib, rux'), we remain focused on the potential room for growth and the long-term opportunity as we view Jakafi in hematologic disorders as fully baked into our valuation and we continue to look for color on the potential to expand the commercial for Jakafi beyond the 2028 loss of exclusivity. On the oncology pipeline, the company highlighted initial clinical activity observed with a CDK2 (cyclin dependent kinase 2) in patients with amplified CCNE1 (cyclin E) with data expected in 2024. We look for guidance on the commercial outlook in 2024 including gross-to-net dynamics for Opzelura and the evolving competitive dynamics for Jakafi following the approval of Ojjaara. We maintain our Neutral with $69 PO.

Additional near-term catalysts include: 1) anticipated approval of axatilimab in 3L+ chronic graft v. host disease (cGVHD) in 2024, 2) initiating phase 3 study of first-line axatilimab in combination with steroids in 2024, 3) initiating phase 2 study of first-line axatilimab in combination with ruxolitinib in 2024, 4) initiating phase 3 study of BETi in combination with rux' in 2H24, 5) proof-of-concept data for zilurgisertib in combo with rux' by mid-24, 6) initiating phase 1 study of JAK2V617F in 1Q24, 7) topline phase 2 data for rux' cream in hidradenitis suppurativa (HS) in 2024, and 8) full phase 2 data for povorcitinib in prurigo nodularis in 1H24.

NBIX: Ph 2 data for '758 in schizo in 2H

Neurocrine Biosciences (NBIX, Buy, $154 PO) highlighted focus on growing the Ingrezza franchise and expansion into neuroendocrinology and neuropsychiatry in 2024. Management has noted they expect to see continued strong growth for Ingrezza sales in 2024 driven by increasing diagnosis rates and physicians' education. The company expects a regulatory decision for the new Ingrezza sprinkle formulation, which would benefit patients who have difficulty swallowing by April 30th. On crinecerfont in congenital adrenal hyperplasia (CAH), the company noted they see a high unmet need and anticipate filing a new drug application in 2024 for the pediatric and adult indications, with an expected approval in 2025. The company highlighted the broad muscarinic portfolio with potential in several neurologic disorders and highlighted topline phase 2 data for NBI-1117568, an M4 agonist, in schizophrenia is expected in 2H24. NBIX has a catalyst-rich 2024 with several additional phase 2 data readouts including: 1) topline phase 2 data for Efmody in adrenal insufficiency and CAH in 1H24, 2) topline phase 2 data for NBI-'845, an AMPA potentiatior, for inadequate response to treatment in Major depressive disorder (MDD) in 1H24, and 3) topline phase 2 data for luvadaxistat, a DAAO inhibitor, in cognitive impairment associated with schizophrenia in 2H24. The company also plans to advance 5 phase 1 programs, including 4 muscarinic compounds and a next-generation VMAT2 inhibitor, in 2024. We continue to be encouraged by the strong commercial performance of Ingrezza and await several catalysts in 2024. We maintain our Buy with $154 PO.

PHVS: FDA clinical hold remains for prophy treatment

Pharvaris (PHVS, Underperform, $11 PO) maintains their focus on progressing their pipeline to develop new therapies for hereditary angioedema (HAE) treatment. Recall, management participated in a productive end-of-phase 2 meeting with the FDA to discuss the trial design for a proposed phase 3 RAPIDe-3 study evaluating PHVS416 for on-demand treatment of HAE. The company has guided that the initiation of the global RAPIDe-3 study is anticipated within 1H24. On the prophylactic HAE program for

PHVS416, we highlight the US clinical hold remains ongoing, but PHVS has submitted a 26-week nonclinical toxicology to the FDA to address regulator concerns. We await more color from management on the outcome of the 26-week study and the timing of a potential resolution. Despite the US clinical hold, the company announced positive topline results for the phase 2 CHAPTER-1 trial evaluating deucrictibant as a prophylactic treatment for HAE (see our Dec 6 note). Although the global prophylactic study achieved its primary endpoint, we view the US clinical hold as significant overhang for the stock and look for a clear resolution path that could add upside to our estimates. We maintain our Underperform rating with $11 PO.

PTCT: Several regulatory pressures upcoming for franchise

PTC Therapeutics (PTCT, Underperform, $20 PO) maintains their focus on following their revised timelines for key pipeline programs. On sepiapterin in phenylketonuria (PKU), a marketing authorization application submission to the European Medicines Agency (EMA) is expected in 1Q24. Recall, the company now needs to complete a 26-week carcinogenicity study before a US regulatory filing and expects to now submit an NDA (new drug application) to the FDA by 3Q24. We remain cautious on initial opportunity given our view that sepiapterin's usage in 1L could be limited as physicians may default to current standard of care options given familiarity. Furthermore, our Key opinion leaders (KOLs) highlighted that payors may require patients to have first tried Kuvan before providing coverage. As such, we currently assign a 75% likelihood-of-success for the program with peak risk-adjusted sales of $480mn in 2032. On Translarna, management has sought re-examination for the recent negative Committee for medicinal products for human use (CHMP) opinion and expects a final decision on full EU approval in Duchenne muscular dystrophy (DMD) in January of 2024 with official European Commission (EC) ratification 67-days after. In addition to future EU Translarna revenues being uncertain, we note the upcoming Emflaza loss of exclusivity in February is another major risk factor for 2024 revenues especially given the FDA recently approved competitor Catalyst Pharmaceutical's (ticker: CPRX) Agamree for DMD (see our Dec 18 note). We await several updates and note high focus on multiple upcoming regulatory pressures in the next 6-12 months, which we think could decide the future of the company. We maintain our Underperform with $20 PO.

Other upcoming catalysts include: 1) follow-up Type C meeting with the FDA for Translarna in DMD in 1Q24, 2) meeting with the FDA and feedback from the EMA on path forward for vatiquinone Friedrich ataxia in 1Q24, 3) interim 12-month data update for PTC518's PIVOT-HD study in Huntington's disease in 2Q24, 4) submit BLA (biologics license application) to FDA in 1Q for Upstaza for the treatment of aromatic l-amino acid decarboxylase deficiency, and 5) topline results for the phase 2 CardinALS trial evaluating utreloxastat in amyotrophic lateral sclerosis expected in 4Q.

RARE: Focus on several late-stage catalysts in 2024

Ultragenyx Pharmaceuticals (RARE, Buy, $85 PO) guides to a busy 2024 with multiple upcoming catalysts for their late-stage rare disease pipeline, including: 1) phase 2 Angelman syndrome data update for at least 20 expansion cohort patients in 1H24, 2) completing phase 3 enrollment for Orbit and Cosmic studies evaluating UX143 in osteogenesis imperfecta (OI) in 1Q and 1H24, respectively. Management also expects to present additional phase 2 data later in 2024, 3) phase 3 data for DTX401 in glycogen storage disease type 1a (GSD1a) in 1H24, 4) interim Stage 1 data evaluating UX701 in Wilson disease in 1H, 5) updated Transfer A data evaluating UX111 for Sanfilippo syndrome at WORLDSymposium in February, and 6) phase 3 enrollment completion for DTX301 in ornithine transcarbamylase (OTC) deficiency in 1H24. We are encouraged by the company's robust pipeline focused on high unmet rare disease indications. We view RARE as a market leader in developing rare disease therapies given their proven track record bringing 4 therapies to market across 5 different indications. We note high focus on the upcoming 2024 pipeline catalysts that if positive could provide substantial upside to the current market price. We highlight RARE as one of our top stock picks of 2024 and maintain our Buy rating with $85 PO.

SAGE: Looking for color on Zurzuvae launch metrics

Sage Therapeutics (SAGE, Neutral, $30 PO) is focused on the launch of Zurzuvae in postpartum depression (PPD) after the drug became commercially available in mid-December. Management has commented they are working with payors on optimizing access to Zurzuvae and increasing awareness, treatment and diagnosis rates in PPD. In our view, the high price ($15.9k) could present challenges for access long-term and we look for color on initial launch metrics. The company is also focusing on advancing SAGE-718 and SAGE-324. SAGE-718, an NMDA (N-methyl-D-aspartate) receptor positive allosteric modulators, is being evaluated in Huntington's disease, Parkinson's disease and Alzheimer's disease. SAGE-324 is being evaluated in the phase 2b KINETIC 2 study in essential tremor with topline data readout expected in mid-2024. Topline data from several phase 2 studies evaluating SAGE-718 are expected in 2024, including: 1) PRECEDENT study in mild cognitive impairment associated (MCI) with Parkinson's disease in early-24 (Q1/Q2), 2) SURVEYOR study in Huntington's disease (HD) cognitive impairment in mid-24 (Q2/Q3), 3) LIGHTWAVE study in MCI and mild dementia due to Alzheimer's disease in late/24 (Q3/Q4) and 4) DIMENTION study in HD cognitive impairment in late-24. In our DCF-based model, we increase our pipeline value to $600mn (prev. $400mn) given the potential for '718 and '324 to expand the commercial opportunity with several trial readouts in 2024. This results in our new $30 PO (prev. $26) We will continue to look for color on expectations for the cadence of the Zurzuvae launch and updates on the pipeline programs and the potential commercial opportunity. We maintain our Neutral with new $30 PO.

Exhibit 2: Summary of key catalysts for some of our covered names
We summarize key 2024 catalysts for some of our covered names

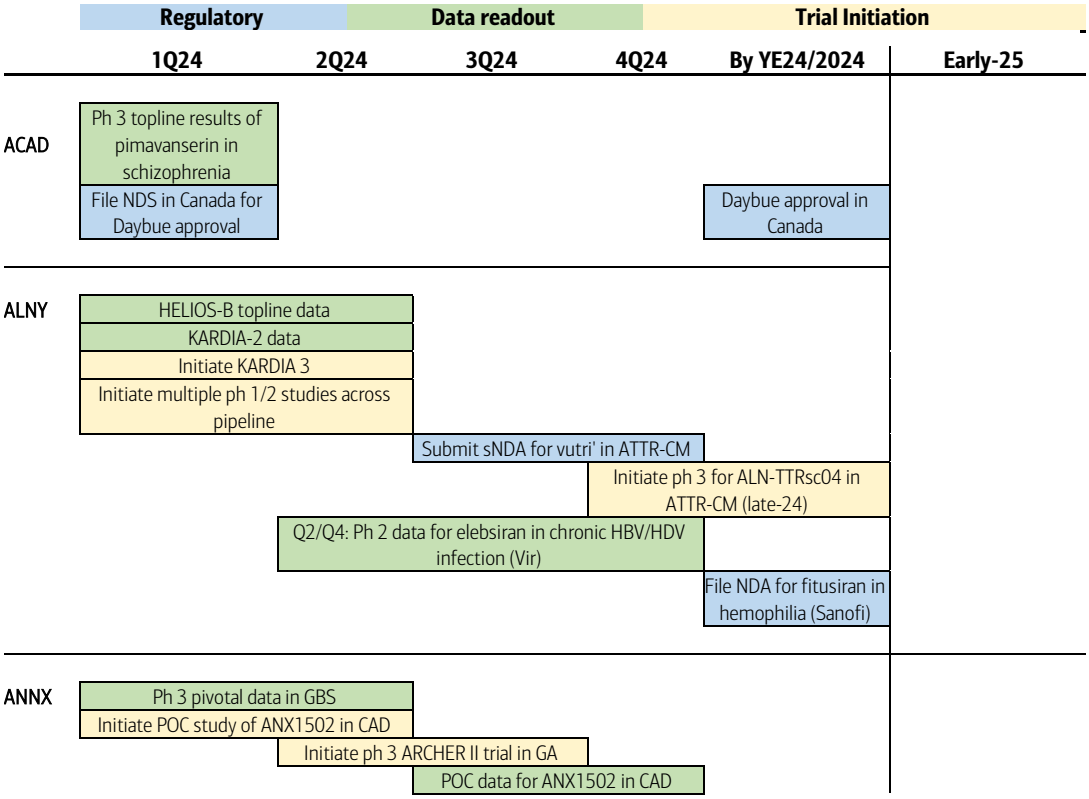


Exhibit 2: Summary of key catalysts for some of our covered names
We summarize key 2024 catalysts for some of our covered names

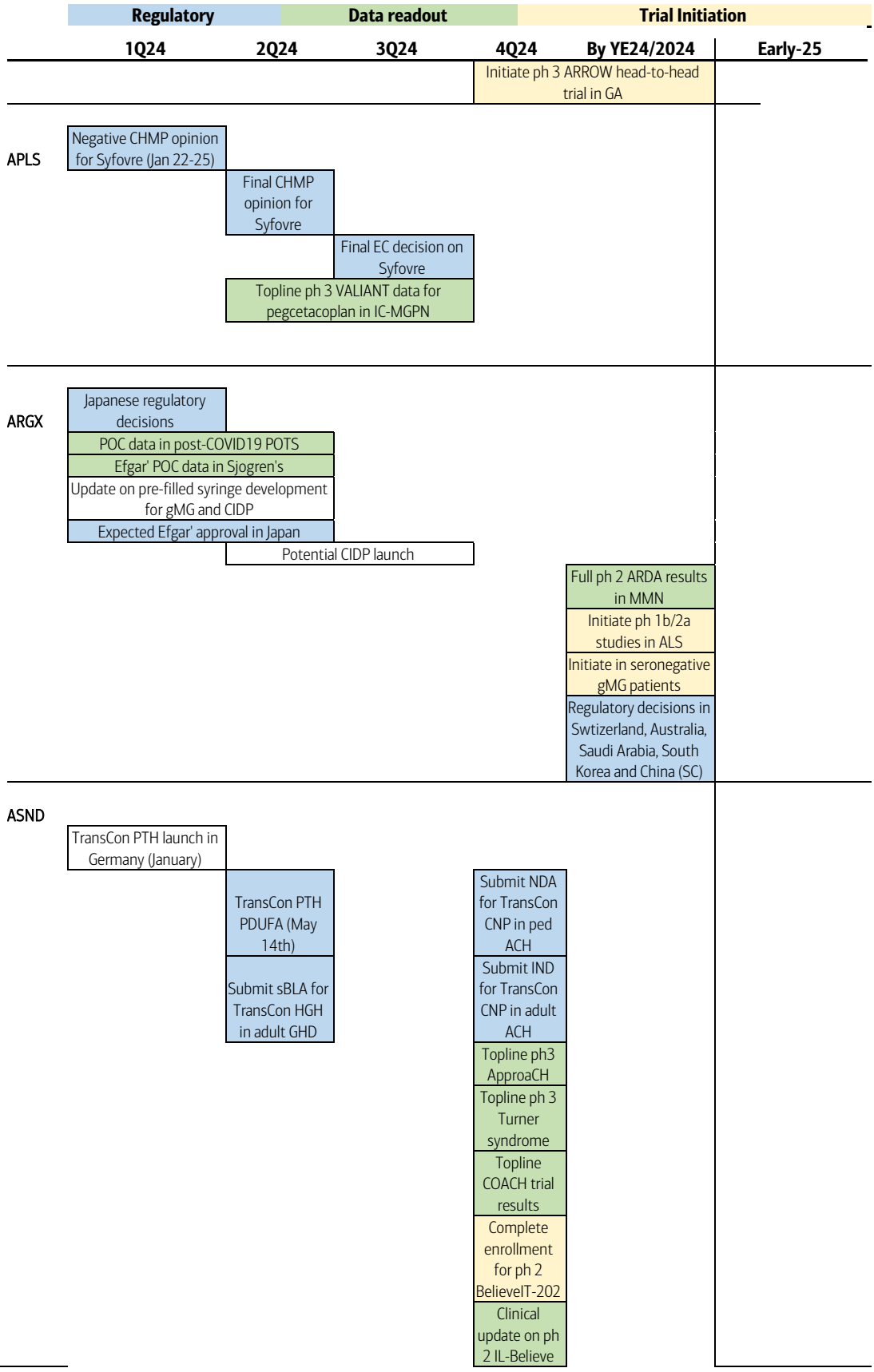


Exhibit 2: Summary of key catalysts for some of our covered names
We summarize key 2024 catalysts for some of our covered names

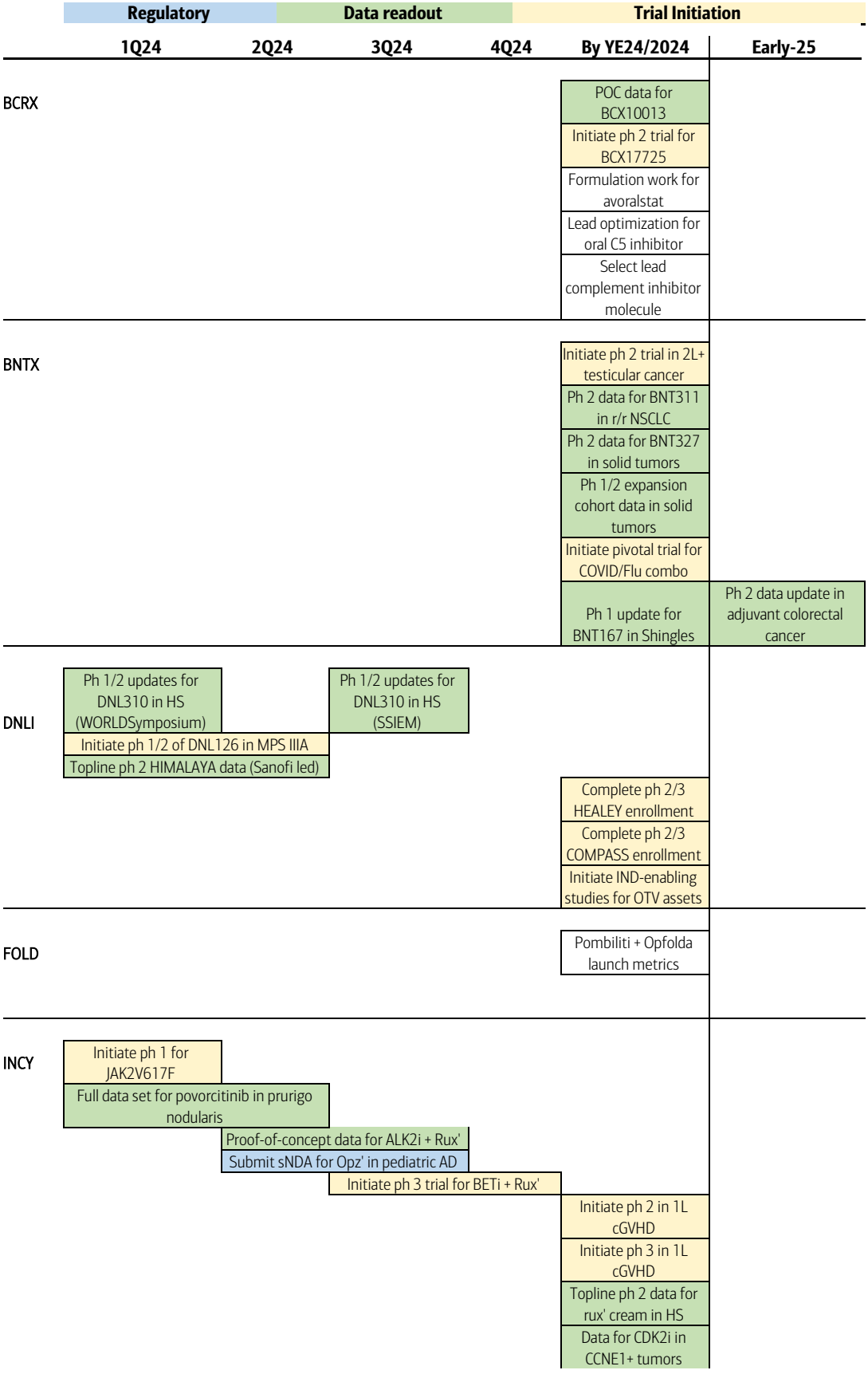


Exhibit 2: Summary of key catalysts for some of our covered names
We summarize key 2024 catalysts for some of our covered names

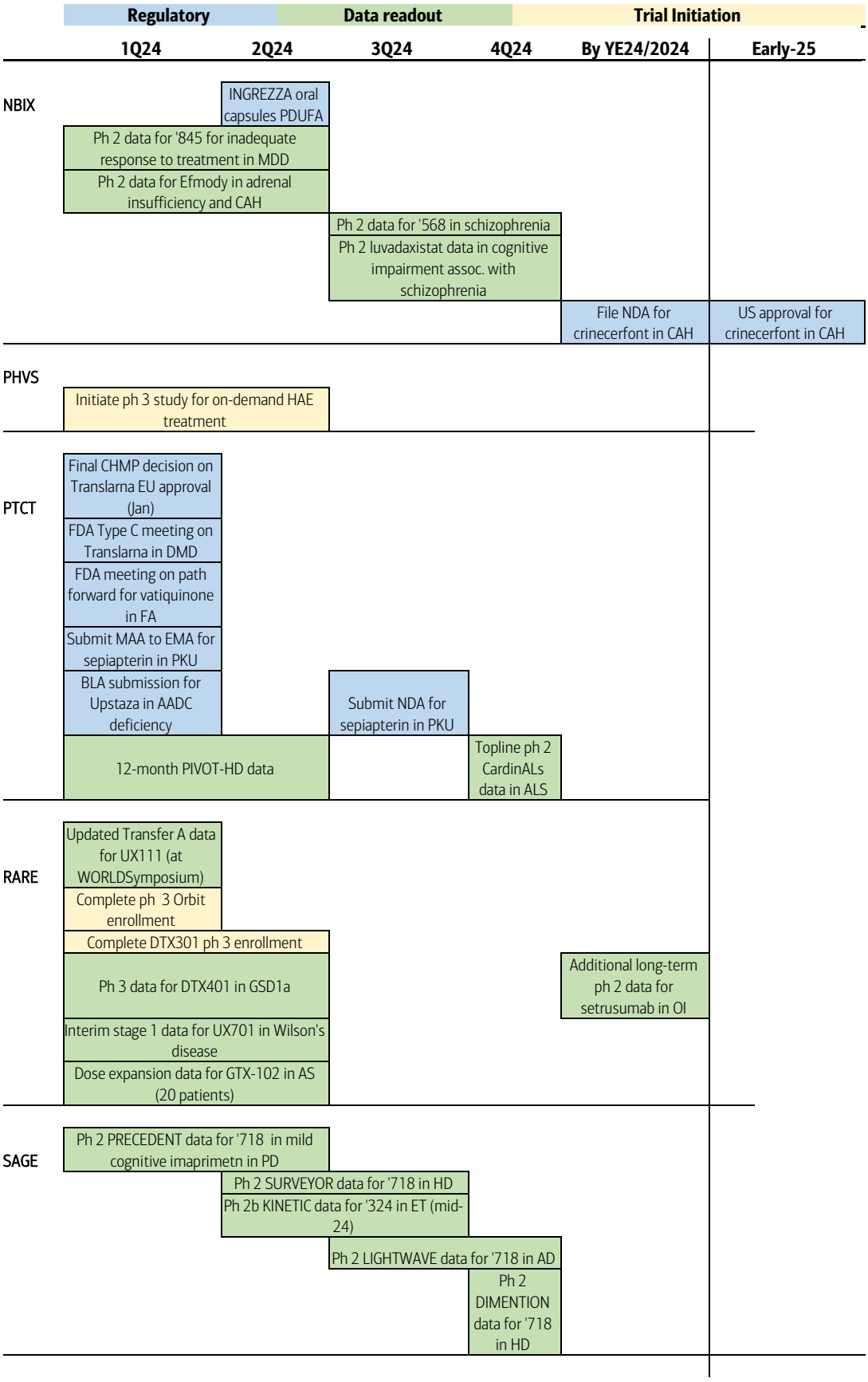
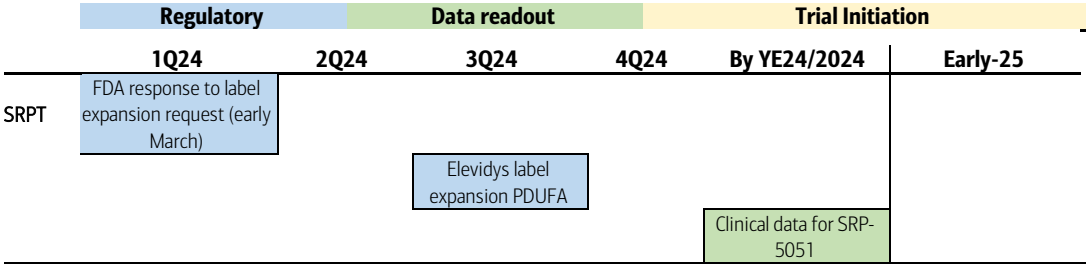


Exhibit 2: Summary of key catalysts for some of our covered names
We summarize key 2024 catalysts for some of our covered names



Abbreviations: 1L: first line, Abs: antibodies, ACH: achondroplasia, AD: Alzheimer's disease, ADP: azlheimer's disease psychosis, aGHVD: acute graft-vs-host disease, ALS: amyotrophic lateral sclerosis, AR: androgen receptor, ALK2i: activin receptor-like kinase-2 inhibitor, AMR: Antibody mediated rejection, ATTR-CM: transthyretin amyloidosis related cardiomyopathy, Axa': axatilimab, Bav: bavdegalutadmide, BETi: bromodomain and extra-terminal inhibitor, BLA: Biologics licence application, CAD: cold agglutinin disease, CAH: congenital adrenal hyperplasia, CAR: chimeric antigen receptor, CHMP: committee for medicinal products for human use, CF: Cystic fibrosis, cCVHD: chonic graft vs. host disease, CIDP: chronic inflammatory demyelinating polyneuropathy, CNP: C-type natriuretic peptide, CTA: clinical trial application, DIPG: diffuse intrinsic pontine glioblastoma, DLBCL: diffuse large B-cell lymphoma, DM1: Myotonic dystrophy type 1, DMD: Duchenne muscular dystrophy, DME: Diabetic macular edema, EC: European Commission, ET: essential tremor, FL: follicular lymphoma, FOS: focal onset seizures, GA: geographic atrophy, gMG: generalized myasthenia gravis, HD: Huntington's disease, HBV: hepatitis B virus, HPV: Human papillomavirus, HSCT-TMA: hematopoietic stem cell transplantation-associated thrombotic microangiopathy, HV: Healthy volunteer, IND: investigational new drug, ITP: Immune thromnocytopenia, mBC: metastatic breast cancer, mCRPC: metastatic castration-resistant prostate cancer, MDAS: Mitocondrial disease associated seizures, MDD: major depressive disorder, MF: myelofibrosis, MMN: Multifocal motor neuropathy, mono: monotherapy, muts: mutations, MSA: multiple system atrophy, NASH: nonalcoholic steatohepatitis, NDA: new drug application, NK: natural killer, nOH: neurogenic orthostatic hypotension, OLE: open label extension, Parsa': parsaclisib, PC-POTS: post COVID postural orthostatic tachycardia syndrome, PD: Parkinson's disease, PDUFA: prescription drug user fee act, ph: phase, PKU: Phenylketonuria, POC: Proof of concept, PPR: postpartum depression, PTH: parathyroid hormone, QD: once daily, RP2D: Recommended phase 2 dose, Rux': ruxolitinib, SBS: Short bowel syndrome, SC: Subcutaneous, Sky: Skytrofa, SOM: severe oral mucositis, ST: solid tumor, T2D: type 2 diabetes, TED: Thryroid eye disease, TNBC: triple negative breast cancer, val': valbenazine, wAMD: wet age-related macular degeneration, yr: year

Source: BofA Global Research

BofA GLOBAL RESEARCH

Exhibit 3: Summary of companies mentioned in this report
Tickers mentioned in this report

Ticker	Company name	QRQ	Price	Price Obj.
ACAD	ACADIA Pharmaceuticals Inc	C29	$28.95	$33
ALNY	Alnylam Pharmaceuticals Inc	B19	$187.49	$246
ANNX	Annexon Inc	C19	$4.41	$6
APLS	Apellis Pharmaceuticals Inc	C19	$66.30	$77
ARGX	Argenx SE	C19	$378.13	$557
ASND	Ascendis Pharma A/S	C19	$133.50	$145
BCRX	BioCryst Pharmaceuticals Inc	C19	$6.09	$11
BNTX	BioNTech SE	C19	$99.57	$159
DNLI	Denali Therapeutics Inc	C19	$18.37	$29
FOLD	Amicus Therapeutics Inc	C19	$12.72	$19
INCY	Incyte Corp	B29	$61.34	$69
NBIX	Neurocrine Biosciences Inc	B19	$132.62	$154
PHVS	Pharvaris NV	C39	$27.85	$11
PTCT	PTC Therapeutics Inc	C39	$28.62	$20
RARE	Ultragenyx Pharmaceutical Inc	C19	$44.00	$85
SAGE	Sage Therapeutics Inc	C29	$26.40	$30
SRPT	Sarepta Therapeutics Inc	C19	$115.39	$164

Source: BofA Global Research, Bloomberg

BofA GLOBAL RESEARCH

Price objective basis & risk

Acadia Pharmaceuticals (ACAD)

Our DCF-derived PO of $33 encompasses commercial drug Nuplazid in PDP at $14/share. Daybue in Rett represents $18/share to our PO. The remainder of our net present value (NPV) comes from pipeline and cash. We use a weighted-average cost of capital (WACC) of 9% for PDP and Rett, consistent with how we value other drugs in similar stages of development in our coverage universe. We assume a 21% tax rate for ACAD and zero terminal value.

Downside risks to our PO are 1) slower-than-expected commercialization of Nuplazid and Daybue, 2) stronger-than-expected competition from other 5HT2A compounds and other drugs in development for the same indications as Nuplazid and Daybue, and 3) negative results in pipeline indications.

Upside risks are 1) faster-than-expected Nuplazid and Daybue uptake in the US, 2) potential for partnerships or transactions with larger pharma companies, and 3) advances of early-stage pipeline assets in pain and central nervous system (CNS) disorders.

Alnylam Pharmaceuticals (ALNY)
Our PO of $246 for ALNY consists of $4/share for Onpattro, $120/share for Vutrisiran, $25/share for Givlaari and Lumasiran, $6/share for Leqvio, $7/share for Fitusiran, $20/share for ALN-AGT, $52/sh for other pipeline and partnered assets and $11/sh for net cash. We apply a WACC of 9% for commercial-stage assets, 10% for Vutrisiran in ATTR-CM, 10% for other late stage pipeline, and 11% for ALN-AGT.

Upside risks to our PO are 1) better-than-expected uptake of its marketed stage assets, 2) approval of Onpattro and Vutrisiran in ATTR cardiomyopathy, 3) early-stage pipeline advancing into late-stage development, and 4) positive results from partnered programs.

Downside risks to our PO are 1) slower-than-expected uptake of commercial assets, 2) unexpected safety in its siRNA-based assets, and 3) higher-than-expected expenses.

Amicus Therapeutics (FOLD)
We use a sum of the parts DCF valuation to arrive at our $19 price objective (PO) for Amicus Therapeutics (FOLD). We value lead asset migalastat Galafold at $10/share using a WACC of 9%, similar to how we value other assets in similar stages of development. Pombiliti in Pompe contributes $11/sh to our DCF derived PO. We value Pombiliti using a 9% WACC. The remainder of our valuation comes from the pipeline and cash, contributing -$2 to our PO. Our DCF goes out to 2035 and we use a zero terminal value, consistent with how we value other companies.

Upside risks to our PO are 1) stronger than expected sales of migalastat in the US, 2) identification of additional amenable mutations treatable by migalastat, 3) stronger than expected launch for Pombiliti, and 5) durability and efficacy data from the early stage gene therapy programs.

Downside risks to our PO are 1) failure of one or more of FOLD's products to reach the market, 2) slower than expected uptake for migalastat, 3) better than expected success for competing marketed and development stage drugs.

Annexon Biosciences (ANNX)
Our price objective (PO) of $6 is based on a probability-adjusted NPV analysis. Our DCF-based valuation for ANNX includes $3/sh for ANX005 in Guillain-Barré syndrome (GBS) and $4/sh for ANX007 in geographic atrophy (GA). The remainder of our valuation comes from pipeline (-$3/sh) and cash ($2/sh). Our model goes out to 2039, with 12% WACC for GBS, 11% WACC for GA, and 14% WACC for the early pipeline.

Upside to our price objective are: 1) accelerated path to regulatory approval, 2) faster-

than-expected enrollment in clinical trials, 3) positive results in clinical trials, 4) wider market penetration than expected.

Downside risks are: 1) negative results in clinical trials, 2) entry or progress of competitors in target indications, 3) failure to replicate GBS Bangladesh clinical trial results in US and EU trials, and 4) lower-than-expected market penetration.

Apellis Pharmaceuticals (APLS)
Our price objective (PO) of $43 is based on a probability-adjusted net present value (NPV) analysis that includes $17/share for peg' in PNH and $64/share for peg' in GA and -$11/share for pipeline/corporate expenses and cash. The remainder of our valuation comes from pipeline and net cash.

Our discounted cash flow (DCF)-based model assumes sales out to 2036 with no terminal growth, with weighted average cost of capital (WACC) of 9% for PNH and GA, and 12% for pipeline.

Upside risks to our PO are 1) better-than-expected penetration in PNH and/or GA, 2) clarity around reports of rare events of occlusive retinal vasculitis following Syfovre injection, 3) less-than-expected neovascularization event in GA in real-world use, 34) faster-than-expected uptake of peg' in GA, and 5) positive data from other complement-related indications, such as C3G, and CAD.

Downside risks to our PO are 1) higher-than-expected neovascularization or occlusive retinal vasculitis events in GA in real-world setting, 2) better-than-expected results from competitors, 3) delay or failure to obtain regulatory approval, and 4) failure to expand into other complement-related indications.

Argenx SE (ARGX)
Our price objective of $557 is based on our DCF-derived model with valuations assigned for efgartigimod in Myasthenia Gravis ($307), Immune Thrombocytopenia ($6), and Chronic Inflammatory Demyelinating Polyneuropathy ($161), pipeline and corporate expenses ($30) and cash. We assign 9% WACC for MG, 10% for ITP, and CIDP. Our DCF valuation is based on estimates out to 2038. We assume peak penetrations of 10%-35% in US depending on the indication.

Upside risks to our PO are (1) better-than-expected efficacy in efgartigimod indications, (2) faster-than-anticipated timeline to approval, (3) additional indications advancing in clinical development, (4) positive data from its partnerships, and (5) higher-than-expected pricing at launch.

Downside risks to our PO are (1) competitors have better-than-expected efficacy, (2) failure to achieve clinically meaningful results in ongoing studies, and (3) unexpected safety events in ongoing trials.

Ascendis Pharma (ASND)
Our $145 price objective for ASND includes $51 for TransCon GH, $82 for TransCon PTH, $9 for oncology assets, and the remainder of value coming from net cash and pipeline. We use a 9% weighted-average cost of capital (WACC) for GH and 10% WACC for PTH, consistent with how we model other drugs in a similar development stage. We assume zero terminal value for all products, also consistent with our valuation of other covered companies.

Risks to our price objective are slower TransCon GH sales, higher-than-expected competition from other long-acting therapies, failure of PTH to advance to commercialization and pushback on pricing from payors.

Biocryst Pharmaceuticals Inc (BCRX)
Our DCF-derived PO of $11 is comprised of $12/share for Orladeyo in preventing HAE attacks, pipeline/corporate expenses, and net cash. We assume a 9% WACC for Orladeyo and 14% WACC for pipeline expenses. We assume no terminal value.

Upside risks to our price objective are 1) Orladeyo uptake faster than we expect, 2) failure of competitor products in development for HAE, 3) increased government funding, and 4) positive outcome from BCX10013 in PNH, C3G and other complement-mediated diseases.

Downside risks to our price objective are 1) slower-than-expected Orladeyo market penetration, 2) high discontinuation rate of Orladeyo, 3) unexpected long term safety concerns, 4) stronger preference for competing products including Takhzyro, and 5) failure of pipeline products, such as BCX10013 in PNH.

BioNTech (BNTX)
Our DCF-derived PO of $159 for BNTX consists of $18/share for FixVac over four indications (melanoma, H&N, prostate) and $5/share for iNeST over four indications (solid tumors). We assign $76/share to the Comirnaty COVID vaccine and -$15/share to BNTX's early-stage pipeline assets including intratumoral immunotherapy, RiboMabs, RiboCytokines and engineered cell therapy and antibody platforms, as well as future potential infectious disease indications. The remaining value in our PO comes from cash. We use 12-14% WACC for FixVac, 12-13% WACC for iNeST, and 8% for BNT162 (COVID vaccine). We also assume 1.5% terminal growth for FixVac and iNeST.

Upside risks to our price objective are 1) approval of COVID vaccine boosters in a broad population, 2) positive data from clinical-stage programs (FixVac and iNeST) in oncology including melanoma and other solid tumors, 3) potential to reach earlier line patient populations based on combination therapies, and 4) positive data from early stage assets from other platforms including cell therapies, antibodies and small molecule immunomodulators.

Downside risks are 1) failure to show benefit in clinical studies, 2) failure to reach optimized turnaround time for iNeST, 3) visibility needed on regulatory path forward for iNeST, 4) competition from other companies pursuing the same therapeutic modalities, and 5) challenges in scaling up to commercial manufacturing capacity.

Denali Therapeutics (DNLI)
Our PO of $29 for DNLI consists of $16/sh for DNL343 in amyotrophic lateral sclerosis (ALS), $4/sh for DNL310 in Hunter syndrome, and $1/sh for DNL151 in Parkinson's disease. $8/sh is contributed from our pipeline assumptions and net cash. We apply a WACC of 11-12% for modeled programs and 14% for pipeline.

Upside risks to our PO are 1) better than expected uptake of modeled programs, 2) positive results from DNL343, DNL310 and DNL151 programs, 3) early-stage pipeline advancing into late-stage development, and 4) positive results from partnered programs.

Downside risks to our PO are 1) slower than expected uptake of modeled programs, 2) unexpected adverse safety issues from the company's transport vehicle (TV) platform technology, 3) clinical trial failures of key pipeline programs, and 4) higher than expected expenses

Incyte Corporation (INCY)
Our PO of $69 for INCY consists of $43/share for Jakafi/Jakavi, $18/share for Ruxolitinib cream, $3/share for Pemazyre, -$2/share for Monjuvi, $4/share for Olumiant royalty, $0.2/share for Iclusig, $1/share for Tabrecta royalty, -$14/share for pipeline, and the remainder in net cash. We apply a weighted-average cost of capital (WACC) of 9% for

commercial-stage assets, 10% for late-stage clinical pipeline, and 11% for earlier-stage clinical pipeline with no terminal value.

Upside risks to our PO are 1) positive data from clinical trials in the pipeline, 2) better than expected results from its marketed assets, 3) additional updates from early-stage assets.

Downside risks to our PO are 1) failure of Jakafi or Opzelura to meet our estimates, 2) business development events that investors view negatively, or 3) negative data in clinical trials.

Neurocrine Biosciences (NBIX)
Our DCF-derived PO of $154 consists of $124/share for Ingrezza for tardive dyskinesia, $6/share for chorea and $8/share for CAH. Royalties from Orilissa for endometriosis and Oriahnn for uterine fibroids represent another $3/share. The rest of the valuation is attributed to cash, corporate expense and pipeline assets. We use a 9% weighted-average cost of capital (WACC) for commercial assets and assume no terminal value, consistent with other companies under coverage that have commercial products, and 13% WACC for pipeline.

Downside risks to our price objective and estimates are Ingrezza not approved for chorea in Huntington's patients, pipeline setbacks, higher-than-expected operating expenses, greater-than-expected competition from other drugs, earlier-than-expected generic competition to NBIX's products, potential for future dilutive cash raises, potential for the US drug pricing environment to worsen and any unexpected management changes.

Pharvaris (PHVS)
Our $11 PO is based on our DCF-based valuation with $8/sh assigned to HAE programs in both prophylaxis and acute on-demand settings. The remainder of our valuation comes from pipeline and corporate expenses, and cash. Our assumptions are based on 12% WACC for the acute on-demand program and prophylaxis program, 6% COGS, 15-20% GTN, and 10%-20% peak penetrations in target indications.

Upside risks to our price objective are: 1) positive data and additional validation in HAE, 2) superior efficacy or safety profile compared to standard of care or other competitors in the pipeline, 3) pipeline expansion to other indications outside of HAE.

Downside risks are: 1) failure to show efficacy in HAE, 2) unexpected side effect, 3) inferior efficacy or safety profile compared to standard of care or other competitors, and 4) competitors entry in HAE.

PTC Therapeutics (PTCT)
Our $20 price objective for PTC reflects $8 for Translarna in DMD, $7 for Evrysdi in SMA, $3 for Emflaza, $8 for GT-AADC, $5 for PKU, and the remainder of our valuation is cash and pipeline spend. We use an 10% WACC for Translarna, a 9% WACC for Emflaza and Evrysdi, and an 10% WACC for Upstaza (PCT-AADC) and sepiapterin, consistent with how we model drugs in similar stage of development. We attach a 14% WACC to the early-stage pipeline pending presentation of data. We assume zero terminal value for all products, also consistent with our valuation of other covered companies.

Downside risks to our price objective are removal of the EU approval for Translarna in DMD, failure to receive approval for Translarna in the US, and slower than expected uptake for Evrysdi in SMA and Upstaza in AADC deficiency.

Upside risks to our price objective are faster-than-expected uptake of Evrysdi in SMA,

higher Emflaza or Translarna sales than expected, US approval for DMD, and a successful AADC launch.

SAGE Therapeutics (SAGE)
Our discounted cash flow (DCF)-derived PO of $30 for SAGE consists of $2/share for Zulresso in PPD. Zuranolone contributes $7/share to our PO for PPD and $4/share in MDD. The remaining value in our PO comes from cash ($14/sh), corporate expenses and pipeline ($1/sh) milestones ($2/sh). We use a 9% weighted-average cost of capital (WACC) for Zulresso, a 9% WACC for Zuranolone in PPD, and 11% WACC in MDD, and assume no terminal value for SAGE.

Upside risks to our price objective are 1) approval of zuranolone in MDD, 2) higher-than-expected penetration of zuranolone in PPD and MDD, and 3) positive data from early stage assets.

Downside risks are 1) failure of Zuranolone in MDD, 2) failure of other clinical programs, and 3) lower-than-expected penetration of zuranolone and Zulresso

Sarepta Therapeutics (SRPT)
Our $164 PO is based on a probability-adjusted net present value (NPV) analysis that includes $17/share for Exondys, $8/share for Vyondys, $22/share for Amondys, $78/share for micro-dystrophin gene therapy, $38/share for LGMD assets. The remainder of our valuation comes from pipeline and cash. Our discounted cash flow (DCF) analysis assumes sales out to 2035, with weighted average cost of capital (WACC) of 9-13%.

Upside risks to our PO are 1) better-than-expected market uptake from its exon skipping assets, 2) SRP-9001 US label expansion, and 3) EU approval of SRP-9001 and other assets.

Downside risks to our PO are 1) failure and delay in approval for exon skipping assets, 2) unexpected safety or durability findings in gene therapy programs, 3) other micro dystrophin competitors, and 4) higher-than-expected royalty payments.

Ultragenyx Pharmaceuticals (RARE)
Our DCF-derived PO of $85 for RARE consists of $10/share for Dojolvi in LC-FAOD, $20/share for Crysvita in XLH and TIO, $0.5/share for Mepsevii in MPS7, $16/share for Angelman, $43/share for gene therapy assets and the remainder for cash and RARE's pipeline. We use a 9% WACC for approved products, 11-12% for clinical-stage products, such as gene therapy programs.

Upside risks to our price objective are: 1) better than expected uptake for its approved products, 2) positive data for clinical stage assets, and 3) accelerated approval for its drug candidates.

Downside risks are: 1) low penetration into rare disease populations, 2) negative data for clinical stage assets, 3) unexpected safety risks associated with clinical stage drug candidates, 4) unexpected generic competition.

Analyst Certification

I, Tazeen Ahmad, hereby certify that the views expressed in this research report accurately reflect my personal views about the subject securities and issuers. I also certify that no part of my compensation was, is, or will be, directly or indirectly, related to the specific recommendations or view expressed in this research report.

US - Biotechnology Coverage Cluster

Investment rating	Company	BofA Ticker	Bloomberg symbol	Analyst
BUY				
	4D Molecular Therapeutics, Inc.	FDMT	FDMT US	Tazeen Ahmad
	Alnylam Pharmaceuticals	ALNY	ALNY US	Tazeen Ahmad
	Amicus Therapeutics	FOLD	FOLD US	Tazeen Ahmad
	Annexon Biosciences	ANNX	ANNX US	Tazeen Ahmad
	Apellis Pharmaceuticals	APLS	APLS US	Tazeen Ahmad
	Argenx SE	ARGX	ARGX US	Tazeen Ahmad
	Arvinas	ARVN	ARVN US	Tazeen Ahmad
	Ascendis Pharma	ASND	ASND US	Tazeen Ahmad
	Biocryst Pharmaceuticals Inc	BCRX	BCRX US	Tazeen Ahmad
	BioNTech	BNTX	BNTX US	Tazeen Ahmad
	Denali Therapeutics	DNLI	DNLI US	Tazeen Ahmad
	Inozyme Pharma, Inc.	INZY	INZY US	Tazeen Ahmad
	Merus	MRUS	MRUS US	Tazeen Ahmad
	Neurocrine Biosciences	NBIX	NBIX US	Tazeen Ahmad
	PepGen Inc	PEPG	PEPG US	Tazeen Ahmad
	Prothena Corporation	PRTA	PRTA US	Tazeen Ahmad
	Rhythm Pharmaceuticals	RYTM	RYTM US	Tazeen Ahmad
	Sarepta Therapeutics	SRPT	SRPT US	Tazeen Ahmad
	Ultragenyx Pharmaceuticals	RARE	RARE US	Tazeen Ahmad
NEUTRAL				
	Acadia Pharmaceuticals	ACAD	ACAD US	Tazeen Ahmad
	Incyte Corporation	INCY	INCY US	Tazeen Ahmad
	SAGE Therapeutics	SAGE	SAGE US	Tazeen Ahmad
UNDERPERFORM				
	Achilles Therapeutics	ACHL	ACHL US	Tazeen Ahmad
	Fate Therapeutics	FATE	FATE US	Tazeen Ahmad
	Fulcrum Therapeutics	FULC	FULC US	Tazeen Ahmad
	Pharvaris	PHVS	PHVS US	Tazeen Ahmad
	PTC Therapeutics	PTCT	PTCT US	Tazeen Ahmad

Disclosures

Important Disclosures

Equity Investment Rating Distribution: Health Care Group (as of 31 Dec 2023)

Coverage Universe	Count	Percent	Inv. Banking Relationships [R1]	Count	Percent
Buy	234	60.94%	Buy	115	49.15%
Hold	80	20.83%	Hold	36	45.00%
Sell	70	18.23%	Sell	29	41.43%

Equity Investment Rating Distribution: Global Group (as of 31 Dec 2023)

Coverage Universe	Count	Percent	Inv. Banking Relationships [R1]	Count	Percent
Buy	1895	53.62%	Buy	1083	57.15%
Hold	832	23.54%	Hold	454	54.57%
Sell	807	22.84%	Sell	383	47.46%

[R1] Issuers that were investment banking clients of BofA Securities or one of its affiliates within the past 12 months. For purposes of this Investment Rating Distribution, the coverage universe includes only stocks. A stock rated Neutral is included as a Hold, and a stock rated Underperform is included as a Sell.

FUNDAMENTAL EQUITY OPINION KEY: Opinions include a Volatility Risk Rating, an Investment Rating and an Income Rating. VOLATILITY RISK RATINGS, indicators of potential price fluctuation, are: A - Low, B - Medium and C - High. INVESTMENT RATINGS reflect the analyst's assessment of both a stock's absolute total return potential as well as its attractiveness for investment relative to other stocks within its Coverage Cluster (defined below). Our investment ratings are: 1 - Buy stocks are expected to have a total return of at least 10% and are the most attractive stocks in the coverage cluster; 2 - Neutral stocks are expected to remain flat or increase in value and are less attractive than Buy rated stocks and 3 - Underperform stocks are the least attractive stocks in a coverage cluster. An investment rating of 6 (No Rating) indicates that a stock is no longer trading on the basis of fundamentals. Analysts assign investment ratings considering, among other things, the 0-12 month total return expectation for a stock and the firm's guidelines for ratings dispersions (shown in the table below). The current price objective for a stock should be referenced to better understand the total return expectation at any given time. The price objective reflects the analyst's view of the potential price appreciation (depreciation).

Investment rating	Total return expectation (within 12-month period of date of initial rating)	Ratings dispersion guidelines for coverage cluster[R2]
Buy	≥ 10%	≤ 70%
Neutral	≥ 0%	≤ 30%
Underperform	N/A	≥ 20%

[R2]Ratings dispersions may vary from time to time where BofA Global Research believes it better reflects the investment prospects of stocks in a Coverage Cluster.

INCOME RATINGS, indicators of potential cash dividends, are: 7 - same/higher (dividend considered to be secure), 8 - same/lower (dividend not considered to be secure) and 9 - pays no cash dividend. *Coverage Cluster* is comprised of stocks covered by a single analyst or two or more analysts sharing a common industry, sector, region or other classification(s). A stock's coverage cluster is included in the most recent BofA Global Research report referencing the stock.

Price Charts for the securities referenced in this research report are available on the Price Charts website, or call 1-800-MERRILL to have them mailed.
BofAS or one of its affiliates acts as a market maker for the equity securities recommended in the report: Acadia Ph, Alnylam, Amicus Therapeutics, Annexon Bios, Apellis, Argenx, Ascendis, Biocryst Pharma, BioNTech, Denali, Incyte, Neurocrine, Pharvaris, PTC Therapeutics, SAGE Therapeutics, Sarepta, Ultragenyx Pharm.
BofAS or an affiliate was a manager of a public offering of securities of this issuer within the last 12 months: Argenx SE, Ultragenyx Pharmaceu.
The issuer is or was, within the last 12 months, an investment banking client of BofAS and/or one or more of its affiliates: Acadia Pharmaceutica, Alnylam Pharmaceutic, Argenx SE, Biocryst, BioNTech, Neurocrine Bioscienc, Ultragenyx Pharmaceu.
BofAS or an affiliate has received compensation from the issuer for non-investment banking services or products within the past 12 months: Acadia Pharmaceutica, Alnylam Pharmaceutic, Annexon Bio, Ascendis Pharma, Biocryst, BioNTech, Denali Therapeutics, Incyte, Pharvaris, Sarepta Therapeutics.
The issuer is or was, within the last 12 months, a non-securities business client of BofAS and/or one or more of its affiliates: Acadia Pharmaceutica, Alnylam Pharmaceutic, Annexon Bio, Ascendis Pharma, Biocryst, BioNTech, Denali Therapeutics, Incyte, Pharvaris, Sarepta Therapeutics.
BofAS or an affiliate has received compensation for investment banking services from this issuer within the past 12 months: Argenx SE, Biocryst, BioNTech, Neurocrine Bioscienc, Ultragenyx Pharmaceu.
BofAS or an affiliate expects to receive or intends to seek compensation for investment banking services from this issuer or an affiliate of the issuer within the next three months: Acadia Pharmaceutica, Alnylam Pharmaceutic, Argenx SE, Biocryst, BioNTech, Neurocrine Bioscienc.
BofAS together with its affiliates beneficially owns one percent or more of the common stock of this issuer. If this report was issued on or after the 9th day of the month, it reflects the ownership position on the last day of the previous month. Reports issued before the 9th day of a month reflect the ownership position at the end of the second month preceding the date of the report: Sarepta Therapeutics.
BofAS or one of its affiliates is willing to sell to, or buy from, clients the common equity of the issuer on a principal basis: Acadia Ph, Alnylam, Amicus Therapeutics, Annexon Bios, Apellis, Argenx, Ascendis, Biocryst Pharma, BioNTech, Denali, Incyte, Neurocrine, Pharvaris, PTC Therapeutics, SAGE Therapeutics, Sarepta, Ultragenyx Pharm.
The issuer is or was, within the last 12 months, a securities business client (non-investment banking) of BofAS and/or one or more of its affiliates: Alnylam Pharmaceutic, Ascendis Pharma, Biocryst, BioNTech, Denali Therapeutics, Incyte, Sarepta Therapeutics.
BofA Global Research personnel (including the analyst(s) responsible for this report) receive compensation based upon, among other factors, the overall profitability of Bank of America Corporation, including profits derived from investment banking. The analyst(s) responsible for this report may also receive compensation based upon, among other factors, the overall profitability of the Bank's sales and trading businesses relating to the class of securities or financial instruments for which such analyst is responsible.

Other Important Disclosures

From time to time research analysts conduct site visits of covered issuers. BofA Global Research policies prohibit research analysts from accepting payment or reimbursement for travel expenses from the issuer for such visits.
Prices are indicative and for information purposes only. Except as otherwise stated in the report, for any recommendation in relation to an equity security, the price referenced is the publicly traded price of the security as of close of business on the day prior to the date of the report or, if the report is published during intraday trading, the price referenced is indicative of the traded price as of the date and time of the report and in relation to a debt security (including equity preferred and CDS), prices are indicative as of the date and time of the report and are from various sources including BofA Securities trading desks.
The date and time of completion of the production of any recommendation in this report shall be the date and time of dissemination of this report as recorded in the report timestamp.

Recipients who are not institutional investors or market professionals should seek the advice of their independent financial advisor before considering information in this report in connection with any investment decision, or for a necessary explanation of its contents.
Officers of BofAS or one or more of its affiliates (other than research analysts) may have a financial interest in securities of the issuer(s) or in related investments.
Refer to BofA Global Research policies relating to conflicts of interest.
"BofA Securities" includes BofA Securities, Inc. ("BofAS") and its affiliates. Investors should contact their BofA Securities representative or Merrill Global Wealth Management financial advisor if they have questions concerning this report or concerning the appropriateness of any investment idea described herein for such investor. "BofA Securities" is a global brand for BofA Global Research.
Information relating to Non-US affiliates of BofA Securities and Distribution of Affiliate Research Reports:
BofAS and/or Merrill Lynch, Pierce, Fenner & Smith Incorporated ("MLPF&S") may in the future distribute, information of the following non-US affiliates in the US (short name: legal name, regulator): Merrill Lynch (South Africa): Merrill Lynch South Africa (Pty) Ltd., regulated by The Financial Service Board; MLI (UK): Merrill Lynch International, regulated by the Financial Conduct Authority (FCA) and the Prudential Regulation Authority (PRA); BofASE (France): BofA Securities Europe SA is authorized by the Autorité de Contrôle Prudentiel et de Résolution (ACPR) and regulated by the ACPR and the Autorité des Marchés Financiers (AMF). BofA Securities Europe SA ("BofASE") with registered address at 51, rue La Boétie, 75008 Paris is registered under no 842 602 690 RCS Paris. In accordance with the provisions of French Code Monétaire et Financier (Monetary and Financial Code), BofASE is an établissement de crédit et d'investissement (credit and investment institution) that is authorised and supervised by the European Central Bank and the Autorité de Contrôle Prudentiel et de Résolution (ACPR) and regulated by the ACPR and the Autorité des Marchés Financiers. BofASE's share capital can be found at www.bofaml.com/BofASEdisclaimer; BofA Europe (Milan): Bank of America Europe Designated Activity Company, Milan Branch, regulated by the Bank of Italy, the European Central Bank (ECB) and the Central Bank of Ireland (CBI); BofA Europe (Frankfurt): Bank of America Europe Designated Activity Company, Frankfurt Branch regulated by BaFin, the ECB and the CBI; BofA Europe (Madrid): Bank of America Europe Designated Activity Company, Sucursal en España, regulated by the Bank of Spain, the

ECB and the CBI; Merrill Lynch (Australia): Merrill Lynch Equities (Australia) Limited, regulated by the Australian Securities and Investments Commission; Merrill Lynch (Hong Kong): Merrill Lynch (Asia Pacific) Limited, regulated by the Hong Kong Securities and Futures Commission (HKSFC); Merrill Lynch (Singapore): Merrill Lynch (Singapore) Pte Ltd, regulated by the Monetary Authority of Singapore (MAS); Merrill Lynch (Canada): Merrill Lynch Canada Inc, regulated by the Canadian Investment Regulatory Organization; Merrill Lynch (Mexico): Merrill Lynch Mexico, SA de CV, Casa de Bolsa, regulated by the Comisión Nacional Bancaria y de Valores; Merrill Lynch (Argentina): Merrill Lynch Argentina SA, regulated by Comisión Nacional de Valores; BofAS Japan: BofA Securities Japan Co., Ltd., regulated by the Financial Services Agency; Merrill Lynch (Seoul): Merrill Lynch International, LLC Seoul Branch, regulated by the Financial Supervisory Service; Merrill Lynch (Taiwan): Merrill Lynch Securities (Taiwan) Ltd., regulated by the Securities and Futures Bureau; BofAS India: BofA Securities India Limited, regulated by the Securities and Exchange Board of India (SEBI); Merrill Lynch (Israel): Merrill Lynch Israel Limited, regulated by Israel Securities Authority; Merrill Lynch (DIFC): Merrill Lynch International (DIFC Branch), regulated by the Dubai Financial Services Authority (DFSA); Merrill Lynch (Brazil): Merrill Lynch S.A. Corretora de Títulos e Valores Mobiliários, regulated by Comissão de Valores Mobiliários; Merrill Lynch KSA Company: Merrill Lynch Kingdom of Saudi Arabia Company, regulated by the Capital Market Authority.

This information: has been approved for publication and is distributed in the United Kingdom (UK) to professional clients and eligible counterparties (as each is defined in the rules of the FCA and the PRA) by MLI (UK), which is authorized by the PRA and regulated by the FCA and the PRA - details about the extent of our regulation by the FCA and PRA are available from us on request; has been approved for publication and is distributed in the European Economic Area (EEA) by BofASE (France), which is authorized by the ACPR and regulated by the ACPR and the AMF; has been considered and distributed in Japan by BofAS Japan, a registered securities dealer under the Financial Instruments and Exchange Act in Japan, or its permitted affiliates; is issued and distributed in Hong Kong by Merrill Lynch (Hong Kong) which is regulated by HKSFC; is issued and distributed in Taiwan by Merrill Lynch (Taiwan); is issued and distributed in India by BofAS India; and is issued and distributed in Singapore to institutional investors and/or accredited investors (each as defined under the Financial Advisers Regulations) by Merrill Lynch (Singapore) (Company Registration No 198602883D). Merrill Lynch (Singapore) is regulated by MAS. Merrill Lynch Equities (Australia) Limited (ABN 65 006 276 795), AFS License 235132 (MLEA) distributes this information in Australia only to 'Wholesale' clients as defined by s.761G of the Corporations Act 2001. With the exception of Bank of America N.A., Australia Branch, neither MLEA nor any of its affiliates involved in preparing this information is an Authorised Deposit-Taking Institution under the Banking Act 1959 nor regulated by the Australian Prudential Regulation Authority. No approval is required for publication or distribution of this information in Brazil and its local distribution is by Merrill Lynch (Brazil) in accordance with applicable regulations. Merrill Lynch (DIFC) is authorized and regulated by the DFSA. Information prepared and issued by Merrill Lynch (DIFC) is done so in accordance with the requirements of the DFSA conduct of business rules. BofA Europe (Frankfurt) distributes this information in Germany and is regulated by BaFin, the ECB and the CBI. BofA Securities entities, including BofA Europe and BofASE (France), may outsource/delegate the marketing and/or provision of certain research services or aspects of research services to other branches or members of the BofA Securities group. You may be contacted by a different BofA Securities entity acting for and on behalf of your service provider where permitted by applicable law. This does not change your service provider. Please refer to the Electronic Communications Disclaimers for further information.

This information has been prepared and issued by BofAS and/or one or more of its non-US affiliates. The author(s) of this information may not be licensed to carry on regulated activities in your jurisdiction and, if not licensed, do not hold themselves out as being able to do so. BofAS and/or MLPF&S is the distributor of this information in the US and accepts full responsibility for information distributed to BofAS and/or MLPF&S clients in the US by its non-US affiliates. Any US person receiving this information and wishing to effect any transaction in any security discussed herein should do so through BofAS and/or MLPF&S and not such foreign affiliates. Hong Kong recipients of this information should contact Merrill Lynch (Asia Pacific) Limited in respect of any matters relating to dealing in securities or provision of specific advice on securities or any other matters arising from, or in connection with, this information. Singapore recipients of this information should contact Merrill Lynch (Singapore) Pte Ltd in respect of any matters arising from, or in connection with, this information. For clients that are not accredited investors, expert investors or institutional investors Merrill Lynch (Singapore) Pte Ltd accepts full responsibility for the contents of this information distributed to such clients in Singapore.

General Investment Related Disclosures:

Taiwan Readers: Neither the information nor any opinion expressed herein constitutes an offer or a solicitation of an offer to transact in any securities or other financial instrument. No part of this report may be used or reproduced or quoted in any manner whatsoever in Taiwan by the press or any other person without the express written consent of BofA Securities.

This document provides general information only, and has been prepared for, and is intended for general distribution to, BofA Securities clients. Neither the information nor any opinion expressed constitutes an offer or an invitation to make an offer, to buy or sell any securities or other financial instrument or any derivative related to such securities or instruments (e.g., options, futures, warrants, and contracts for differences). This document is not intended to provide personal investment advice and it does not take into account the specific investment objectives, financial situation and the particular needs of, and is not directed to, any specific person(s). This document and its content do not constitute, and should not be considered to constitute, investment advice for purposes of ERISA, the US tax code, the Investment Advisers Act or otherwise. Investors should seek financial advice regarding the appropriateness of investing in financial instruments and implementing investment strategies discussed or recommended in this document and should understand that statements regarding future prospects may not be realized. Any decision to purchase or subscribe for securities in any offering must be based solely on existing public information on such security or the information in the prospectus or other offering document issued in connection with such offering, and not on this document.

Securities and other financial instruments referred to herein, or recommended, offered or sold by BofA Securities, are not insured by the Federal Deposit Insurance Corporation and are not deposits or other obligations of any insured depository institution (including, Bank of America, N.A.). Investments in general and, derivatives, in particular, involve numerous risks, including, among others, market risk, counterparty default risk and liquidity risk. No security, financial instrument or derivative is suitable for all investors. Digital assets are extremely speculative, volatile and are largely unregulated. In some cases, securities and other financial instruments may be difficult to value or sell and reliable information about the value or risks related to the security or financial instrument may be difficult to obtain. Investors should note that income from such securities and other financial instruments, if any, may fluctuate and that price or value of such securities and instruments may rise or fall and, in some cases, investors may lose their entire principal investment. Past performance is not necessarily a guide to future performance. Levels and basis for taxation may change.

This report may contain a short-term trading idea or recommendation, which highlights a specific near-term catalyst or event impacting the issuer or the market that is anticipated to have a short-term price impact on the equity securities of the issuer. Short-term trading ideas and recommendations are different from and do not affect a stock's fundamental equity rating, which reflects both a longer term total return expectation and attractiveness for investment relative to other stocks within its Coverage Cluster. Short-term trading ideas and recommendations may be more or less positive than a stock's fundamental equity rating.

BofA Securities is aware that the implementation of the ideas expressed in this report may depend upon an investor's ability to "short" securities or other financial instruments and that such action may be limited by regulations prohibiting or restricting "shortselling" in many jurisdictions. Investors are urged to seek advice regarding the applicability of such regulations prior to executing any short idea contained in this report.

Foreign currency rates of exchange may adversely affect the value, price or income of any security or financial instrument mentioned herein. Investors in such securities and instruments, including ADRs, effectively assume currency risk.

BofAS or one of its affiliates is a regular issuer of traded financial instruments linked to securities that may have been recommended in this report. BofAS or one of its affiliates may, at any time, hold a trading position (long or short) in the securities and financial instruments discussed in this report.

BofA Securities, through business units other than BofA Global Research, may have issued and may in the future issue trading ideas or recommendations that are inconsistent with, and reach different conclusions from, the information presented herein. Such ideas or recommendations may reflect different time frames, assumptions, views and analytical methods of the persons who prepared them, and BofA Securities is under no obligation to ensure that such other trading ideas or recommendations are brought to the attention of any recipient of this information.

In the event that the recipient received this information pursuant to a contract between the recipient and BofAS for the provision of research services for a separate fee, and in connection therewith BofAS may be deemed to be acting as an investment adviser, such status relates, if at all, solely to the person with whom BofAS has contracted directly and does not extend beyond the delivery of this report (unless otherwise agreed specifically in writing by BofAS). If such recipient uses the services of BofAS in connection with the sale or purchase of a security referred to herein, BofAS may act as principal for its own account or as agent for another person. BofAS is and continues to act solely as a broker-dealer in connection with the execution of any transactions, including transactions in any securities referred to herein.

BofA ESGMeter Methodology:

ESGMeter is a proprietary metric based on quantitative analysis and fundamental analyst inputs that reflects our assessment of a company's Environmental, Social and Governance-related attributes. The ESGMeter is intended to indicate a company's likelihood of experiencing stronger financial stability (higher return on equity and lower earnings and price volatility) over the next three years relative to peer group. There are three ESGMeter levels - Low, Medium, and High - which indicate whether a company has attributes most likely to translate into superior financial stability (in the case of a High level) or weaker financial stability (in the case of a Low level) over the next three years relative to its peer group. A Medium level suggests that a company exhibits ESG characteristics that are likely associated with financial stability results in line with its peer group over the next three years. Full details of our methodology, financial stability definition and disclaimers are available at BofA ESGMeter methodology. ESGMeter is not indicative of a company's future stock price performance and is not an investment recommendation or rating. ESGMeter is independent of the BofA Global Research fundamental equity analyst's investment rating, volatility risk rating, income rating or price objective for that company.