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BUYSIDE VIEW XXIII

MILESTONES GALORE

BY STEPHEN HANSEN, ASSOCIATE EDITOR AND JENNIFER RHODES, STAFF WRITER

The maturation of the biotech sector over the past few years finds fund managers focusing on a slew of clinical milestones and commercial launches in 2015, but only a handful of approvals.

Buysiders told BioCentury that 2014 was almost an ideal year for investing in biotech, as new drugs were approved at a phenomenal rate, drug launches were setting records and there were only a handful of notable clinical failures.

Indeed, **FDA** approved 41 new drugs last year, with a first cycle approval rate of 78% — tied with 2013 for the all-time high in first-cycle approvals. The influx of new drugs will have investors watching the launches of potential sector growth drivers closely, such as the first two mAbs against PD-1, Opdivo nivolumab from **Bristol-Myers Squibb Co.** and **Ono Pharmaceutical Co. Ltd.**, and Keytruda pembrolizumab from **Merck & Co. Inc.**

They'll also be watching HCV launches as **AbbVie Inc.** and **Gilead Sciences Inc.** engage in a price war sparked by pharmacy benefits managers looking to cut costs. Next up for investors are the anticipated summer launches of PCSK9 inhibitors from **Amgen Inc.** and partners **Regeneron Pharmaceuticals Inc.** and **Sanofi**.

But while the new commercial drivers are important for maintaining sector momentum, investors said 2015 is mostly about clinical execution. "It is a data-driven year," OrbiMed's Sven Borho told BioCentury.

One of the more anticipated clinical milestones of the year has already occurred, as **Biogen Idec Inc.** last week disclosed mixed top-line Phase II data in optic neuritis for its BIIB033 antibody against LINGO-1.

Cancer immunotherapy remains the hottest therapeutic area headed into 2015. But unlike 2014, which was more focused on the PD-1s, the story in 2015 will be the beginnings of a shakeout around which cancer immunotherapy combinations work best as clinical data are reported.

Non-alcoholic steatohepatitis (NASH) and rare diseases will continue as areas of interest for investors in 2015.

In addition, buysiders said two other areas will be garnering more investor attention this year: antibiotics and gene therapy. While investors don't expect to see major commercial or regulatory news in gene therapy in 2015, multiple clinical proof-of-concept (POC) milestones could fuel a growing momentum in the space.

LAUNCH PAD

With the high number of 2014 drug approvals, investors are hoping the newly marketed therapies can continue to drive sector performance like Gilead's Sovaldi sofosbuvir and Biogen Idec's multiple sclerosis drug Tecfidera dimethyl fumarate did in 2014.

Sovaldi set a record for a drug launch with nine-month sales of $8.5 billion, while Tecfidera had $2 billion in sales over the same period.

Borho said the Opdivo launch "has all the chances of being bigger than Tecfidera. It is obviously not going to beat Sovaldi/Harvoni, but it has better longevity." apo Asset Management's Kai Brüning agreed, saying the PD-1 launches should drive generalist interest in the sector.

Keytruda numbers are expected in Merck's 4Q14 earnings on Feb. 4. The mAb was launched in September, and analysts have not yet weighed in with sales estimates.

Opdivo was approved for advanced melanoma in late December, so Bristol-Myers isn't likely to report sales figures until its 1Q15 earnings in April. Analysts have not provided estimates for Opdivo sales yet, either.

Investors will also continue to follow the HCV pricing wars in which pharmacy benefit managers (PBMs) are using their considerable purchasing power to negotiate discounts for the two approved interferon-free combination therapies: AbbVie's Viekira Pak ombitasvir/paritaprevir/ritonavir/dasabuvir and Gilead's Harvoni ledipasvir/sofosbuvir.

In December, AbbVie agreed to an exclusive deal with **Express Scripts Holding Co.** for Viekira Pak that essentially cut out Gilead's HCV drugs Sovaldi and Harvoni. Last week, Gilead added two deals of its own. On Jan. 5, Gilead announced an exclusive deal for Harvoni and Sovaldi with the second-largest U.S. PBM, **CVS Health Corp.** Then on Jan. 8, **Anthem Inc.** agreed to make Harvoni its preferred drug to treat HCV across its employer-based plans.

A few investors worried that the tack taken by PBMs in HCV could also be applied to the PCSK9s. Investors felt Amgen's evolocumab and alirocumab from Regeneron and Sanofi had shown little differentiation in Phase III testing, and so could potentially be played against each other.

Clearbridge's Marshall Gordon said while the PBMs got caught flat-footed in HCV, the PCSK9s "are on their radar screen."

Evolocumab has an Aug. 27 PDUFA date. Sanofi's submission of a BLA for alirocumab is expected early this year, and the partners have said they will use a Priority Review voucher for the alirocumab BLA.

IMMUNOTHERAPY COMBO SHAKEOUT

In cancer immunotherapy, buysiders expect to see the start of a shakeout in terms of which combination therapies work best in the space.

Keytruda is being tested in combination trials with at least 12 other targeted agents; Bristol-Myers is testing at least 10 different agents with Opdivo across 16 different trials; and **Genentech Inc.** and **AstraZeneca plc**'s **MedImmune LLC** unit are testing their respective PD-L1 mAbs in combination with at least eight different targeted therapies.

"It is going to be exciting because we'll see a lot of the combination data coming through for cancer immunotherapy," LSP's Joep Muijrers told BioCentury.

Polar Capital's David Pinniger added 2015 also will show which particular diseases "represent the first wave of opportunities" for combinations.

Investors said one of the more anticipated sets of data will be for Opdivo plus Yervoy ipilimumab. At least one trial, the Phase II CheckMate 069 trial in metastatic melanoma, is expected to read out in 2015.

"IT IS A DATA-DRIVEN YEAR."

SVEN BORHO, ORBIMED

Pinniger and other investors were quick to add that cancer immunotherapy was still in the relatively early stages of development, with years left to run. Although the PD-1/PD-L1 assets are held by pharma, biotech specialist investors are interested in the products in part because combinations will create partnering and acquisition opportunities for biotechs that can contribute molecules to the cocktails.

Beyond the mAbs against PD-1 or PD-L1, the most popular pick among specialist investors was **Innate Pharma S.A.** The French biotech's lead program is lirilumab, a human mAb against killer cell immunoglobulin-like receptors (KIRs) that is partnered with BMS. Investors expect Phase II data for monotherapy in acute myelogenous leukemia (AML) by year end. The mAb is also being studied in multiple Phase I trials in combination with either Opdivo or Yervoy to treat various cancers.

Incyte Corp. is another name investors will be watching in cancer immunotherapy. The company has deals to test its INCB24360 in combination trials with mAbs against PD-1 or PD-L1 from Genentech, Merck, MedImmune and Bristol-Myers. INCB24360 is an indoleamine 2,3-dioxygenase 1 (IDO1) inhibitor that is in Phase II testing for ovarian cancer and in Phase I/II testing to treat metastatic melanoma.

Incyte added to its cancer immunotherapy stable on Jan. 9 when it announced a deal with **Agenus Inc.** to develop preclinical mAbs against four immune checkpoint targets. Agenus received $60 million up front in cash and an equity investment and is eligible for up to $350 million in milestones, plus royalties.

While Incyte was a popular pick, Pinniger said he was slightly wary of the stock because of the high valuation and uncertainty about how much of the value is attributed to the IDO1 program. Incyte was up 44% in 2014 and finished the year with a market cap of $12.3 billion. The company has

2015 milestones for non-cancer programs including baricitinib, where additional Phase III data in rheumatoid arthritis (RA) and Phase II data in diabetic nephropathy are expected this year. The JAK-1 and JAK-2 inhibitor is partnered with **Eli Lilly and Co.**

Medical Strategy's Harald Schwarz picked **Celldex Therapeutics Inc.** In November the biotech jumped nearly 30% after it reported an overall survival benefit for rindopepimut in a Phase II trial of EGFRvIII-positive glioblastoma. Additional Phase II data for the vaccine targeting EGFR variant III are expected this year. Additionally, Celldex is conducting various Phase I/II trials of varlilumab, a mAb targeting CD27, in combination with Opdivo, Yervoy and other cancer immunotherapies.

CAR CRAZY

Investors also said they'd be closely following chimeric antigen receptor (CAR) T cell therapies.

Deerfield's Bill Slattery said **Juno Therapeutics Inc.** and **Kite Pharma Inc.** "are names we should all be watching and paying close attention to, as they have truly transformational potential."

"NASH WILL BE ONE OF THE HOT THERAPEUTIC AREAS GOING FORWARD."

LINDEN THOMSON, AXA-FRAMLINGTON

On the back of data showing complete responses of 70% or more in hematological malignancies for their respective CAR T programs, both companies were strong performers in 2014. Juno raised $304.2 million in an IPO the week before Christmas for a postmoney valuation of $1.9 billion and finished the year up more than 115% with a market cap of $4.2 billion.

Kite started off 2015 with a big deal with Amgen that included a $60 million upfront payment, with each partner eligible for $525 million in milestones for programs developed by the other partner. The companies will use Kite's engineered autologous cell therapy (eACT) platform to develop chimeric antigen receptor (CAR) T cell therapies against Amgen's cancer targets (see BioCentury Extra, Jan. 5, 2015).

Kite raised $146.6 million in an IPO in June, and in December parlayed encouraging Phase I data for KTE-C19 in acute lymphoblastic leukemia (ALL) into a bumped-up $216.4 million follow-on. Kite finished the year up 239% with a market cap of $2.4 billion.

Both Muijrers and Pinniger noted that while the CAR T therapies can be powerful, their safety profiles will be followed closely. Pinniger cited a presentation of data at the **American Society of Hematology** (ASH) meeting in December in which treatment with **Novartis AG**'s CTL019 CAR T cell therapy resulted in almost every patient experiencing cytokine release syndrome.

"Those wrinkles need to be worked out," Pinniger said.

Muijrers and Pinniger named **Cellectis S.A.** as a pick in the CAR T space. The company's allogeneic CAR T cell therapy, UCART19, is in preclinical testing, and in early January the company said it plans to add a listing in the U.S.

Slattery noted investors will be looking for CAR T companies to create value by showing not only durable responses in hematological malignancies, but also how well the technology can migrate into solid tumors.

MM AND MORE

Although cancer immunotherapy was the dominant topic among investors, buysiders still are anticipating plenty of other milestones in cancer. One of those other hot areas is multiple myeloma, where investors said approval of **Celgene Corp.**'s Revlimid lenalidomide as maintenance therapy for MM is important. The application has a Feb. 22 PDUFA date.

In the clinic, investors have been primarily focused on mAbs against CD38. Multiple buysiders said they expect to see the first clinical data for MOR202 from partners **MorphoSys AG** and Celgene. These data will likely be closely compared to results for daratumumab from **Genmab A/S** and **Johnson & Johnson**, which is in Phase III testing and has breakthrough therapy designation for relapsed or refractory MM. Genmab is a pick for HBM Partners' Ivo Staijen, as he thinks the partners might submit a regulatory application or even possibly get approval for daratumumab in MM in 2015.

G2 Investment's Julia Balanova named **Karyopharm Therapeutics Inc.** as a pick in MM. Last June, the company's stock nearly doubled after announcing Phase I data for selinexor in MM. This half, Karyopharm is expected to start a pivotal trial of the oral selective inhibitor of nuclear export (SINE) protein exportin 1 (XPO1; CRM1) to treat MM.

Other areas of interest in cancer included the cyclin dependent kinase 4 (CDK4) and CDK6 inhibitors in breast cancer. Janus Capital's Ethan Lovell said he expects this class of agents to generate quite a bit of enthusiasm. **Pfizer Inc.**'s palbociclib is the most advanced; it is under review with an April 13 PDUFA date. Pfizer has rights to the compound from Onyx Pharmaceuticals Inc., now part of Amgen.

Lovell said he's also anticipating Phase II data for Novartis' LEE011 and Lilly's abemaciclib.

DASH IN NASH

While cancer immunotherapy is the headline in 2015, Investors said NASH will continue to attract investors — and a Jan. 6 deal between Gilead and **Phenex Pharmaceuticals AG** shows why.

Gilead acquired a farnesoid X receptor (FXR; NR1H4) program from Phenex for an upfront payment and development milestones that could total $470 million. The deal includes FXR agonists Px-104 and Px-102. Px-104, the most advanced molecule, is in Phase II testing for non-alcoholic fatty liver disease (NAFLD) to establish a dose regimen for a Phase IIb trial to treat NASH.

Even before the deal was announced, investors told BioCentury they were looking out for Gilead to report Phase II data in NASH for simtuzumab by mid-year. The humanized mAb against lysyl oxidase-like 2 (LOXL2) also is in Phase II testing for other fibrotic diseases including idiopathic pulmonary fibrosis (IPF) and liver fibrosis.

"NASH will be one of the hot therapeutic areas going forward," AXA-Framlington's Linden Thomson told BioCentury.

Pinniger said he's been buying **Intercept Pharmaceuticals Inc.** on share price weakness over the past six months. In January 2014 the biotech reported positive data for obeticholic acid in the Phase II FLINT trial, causing the stock to soar more than 500% in the days following the announcement. The stock has declined since then, which some buysiders said could be due to the uncertainty about the design of a Phase III trial. One buysider who asked not to be named told BioCentury, "A lot of people really want to see exactly what the endpoint will be, what the FDA has agreed to."

Intercept still finished the year up 128% with a market cap of $3.3 billion. Pinniger said the stock could take off again if the company is able to run its Phase III without a pre-approval CV outcomes study.

Pinniger, Mann Bioinvest's Andy Smith and Lovell all named **Genfit S.A.** as a pick in NASH. This quarter investors expect to see Phase IIb NASH data for GFT505, a dual PPAR alpha and delta agonist.

Another small cap NASH pick was **Conatus Pharmaceuticals Inc.** The company's emricasan is in Phase II testing, with data expected this quarter. A separate Phase II trial in cirrhosis is expected to report data in 2H15. Emricasan is a pan-caspase inhibitor.

ORPHAN DISEASES

Investors see Orphan diseases remaining hot in 2015, given the pricing power the drugs can command. Numerous large caps led the buysiders' picks, with **BioMarin Pharmaceutical Inc.** the most popular choice.

ClearBridge's Gordon said he's anticipating Phase II data for BMN 111, an analog of C-type natriuretic peptide (CNP; NPPC) to treat achondroplasia in 2Q15. Other investors are looking for Phase III data in 4Q15 for PEG PAL to treat phenylketonuria. Gordon and Staijen noted the pending acquisition of **Prosensa Holding N.V.** and its Duchenne muscular dystrophy (DMD) assets are a potential upside for BioMarin.

"I think the Prosensa acquisition was rather cheap," Staijen said.

He noted Prosensa's programs have the potential to cover a wide range of DMD mutations, while competitor **PTC Therapeutics Inc.**'s Translarna ataluren, which is approved in Europe to treat nonsense mutation DMD, covers about 10-15% of the patient population. Yet PTC is valued at about $1.6 billion vs. the $680 million up front price tag for Prosensa. "That tells you that BioMarin made a good deal, if you assume the Prosensa drug will be approved," he said.

Prosensa expects to complete a rolling submission of an NDA for drisapersen for DMD in 1Q15. For PTC, investors are also looking for Phase III data in mid-2015 for Translarna in DMD.

Pinniger said he is looking for Phase IIb data for **Vertex Pharmaceuticals Inc.**'s Kalydeco ivacaftor plus VX-661 for cystic fibrosis (CF) patients with two copies of the delta F508 mutation, which are expected in early 2015. He added that CF might begin to open up a bit more in 2015 with early data coming from other players like **ProQR Therapeutics N.V.**

"Cystic fibrosis is not a space that strategics are going to ignore for too long," he said.

Omega Fund's Otello Stampacchia, Staijen and Bogan Associates' Andrew Bogan named **Alnylam Pharmaceuticals Inc.** as an Orphan pick for 2015. The company plans to start clinical trials of three new molecules in 2015, and will have interim Phase I/II data for ALN-CC5, an RNAi targeting complement 5 (C5) for paroxysmal nocturnal hemoglobinuria (PNH).

Alexion Pharmaceuticals Inc. is a 2015 pick for Staijen, as he anticipates approval of asfotase alfa, which is under review in Europe and the U.S. to treat hypophosphatasia (HPP). Staijen also likes **Synageva BioPharma Corp.** He expects the company to receive approval for sebelipase alfa to treat lysosomal acid lipase (LAL) deficiency. The compound has breakthrough therapy designation for the indication in the U.S.

Investors took different sides on a race to bring a new therapy to market for hyperkalemia. Gordon and Pinniger picked the most advanced company, **Relypsa Inc.**, whose oral potassium binder patiromer is under review in the U.S. with an Oct. 21 PDUFA date.

Schwarz's pick for 2015 is competitor **ZS Pharma Inc.**, which in September reported positive Phase III data for sodium zirconium cyclosilicate and expects to submit regulatory applications this half. "Even if both the Relypsa and ZS Pharma drugs are approved, I think there's enough space for them both to get a nice market share," Medical Strategy's Mario Linimeier said.

"CYSTIC FIBROSIS IS NOT A SPACE THAT STRATEGICS ARE GOING TO IGNORE FOR TOO LONG."

DAVID PINNIGER, POLAR CAPITAL

GENE THERAPY

While investors don't expect gene therapy to become a mainstay therapeutic option by the end of 2015, most said what used to be a curse word among buysiders is now on all their radar screens. Multiple opportunities to generate additional clinical POC in 2015 could make the already interesting space even hotter as the year progresses, investors said.

"Gene therapy is one area we've seen real innovation," Slattery said. "It could be a wonderful year for gene therapy."

While investors acknowledged there are a few leading names like **uniQure N.V.**, whose Glybera alipogene tiparvovec is the only approved gene therapy, a few buysiders like Thomson and Brüning said there hasn't been enough differentiation yet to pick the winners. Thus, said Thomson, "I've approached it by holding almost a basket of gene therapy companies. It is certainly a space that is coming of age."

And while Stampacchia said gene therapy might not be ready for prime time in terms of getting drugs approved, it is an area that is capturing the imagination of generalist investors.

The leading gene therapy picks were uniQure and **bluebird bio Inc.** In December, bluebird jumped 72% after reporting interim Phase I/II data for its LentiGlobin BB305 gene therapy that showed two patients with beta thalassemia major had been free from the need for transfusions for three and five months following treatment. Investors expect additional

Phase I/II data this year, as well as Phase I data for the gene therapy in sickle cell disease.

uniQure, **Baxter International Inc.**, and **Spark Therapeutics Inc.** have gene therapies in Phase I/II testing to treat hemophilia, a disease area investors are following. Gordon and Staijen are also interested in BioMarin's BMN 270, which is in Phase I testing.

Spark, which filed for an IPO in December, also partnered with Pfizer last month to develop gene therapies for hemophilia B. But Spark's lead program, SPK-RPE65, is what Gordon thinks may really drive interest in the IPO. In 2H15 the biotech expects to report Phase III data for the gene therapy to treat inherited retinal dystrophies, including Leber's congenital amaurosis (LCA). He noted this would be the first Phase III data for a gene therapy in the eye.

Pinniger and Lovell named **Celladon Corp.** as a potential breakout candidate for 2015. The company expects to report Phase IIb data in April for its Mydicar gene therapy to treat heart failure.

"If this Phase IIb data comes through positive, it could be a monster stock," Pinniger said.

Staijen is also anticipating Phase IIa data in mid-2015 for **Avalanche Biotechnologies Inc.**'s AVA-101 to treat wet age-related macular degeneration (AMD).

ANTIBIOTICS

Almost every buysider said they'll be looking to invest in antibiotics plays in 2015. Gordon said it is one of the cheapest areas in biotech: "In antibiotics you can get late-stage companies with good potential at reasonable valuations."

He said he viewed antibiotics as less risky, because "Phase II data for an antibiotic is much more predictive than Phase II data for a cancer drug."

Muijrers and Thomson agreed, while Stampacchia said there is now a commercial opportunity for antibiotic programs that focus on narrow patient populations with severe infections. Staijen noted antibiotics companies are also prime takeout targets.

The two most popular picks were **Cempra Inc.** and **Tetraphase Pharmaceuticals Inc.**

Last week, Cempra met investor expectations by announcing positive Phase III data for oral solithromycin in community acquired bacterial pneumonia (CABP). A second Phase III study of intravenous solithromycin is ongoing. Muijrers noted that because the oral study was positive, the IV formulation has a strong likelihood of working as well.

Tetraphase has its own Phase III readout this year, as investors expect data for eravacycline from the Phase III IGNITE 2 trial to treat complicated urinary tract infections (cUTIs) in mid-2015.

Schwarz picked **Achaogen Inc.** in antibiotics because the biotech has taken the unusual approach of conducting a superiority trial in Phase III. Linimeier said interim Phase III data for plazomicin to treat carbapenem-resistant bacterial infections could come as early as 2H15.

Paratek Pharmaceuticals Inc. also garnered mentions from several buysiders, as the company plans to start Phase III testing this half for omadacycline to treat CABP and acute bacterial skin and skin structure infections (ABSSSIs).

> **"IN ANTIBIOTICS YOU CAN GET LATE-STAGE COMPANIES WITH GOOD POTENTIAL AT REASONABLE VALUATIONS."**
>
> MARSHALL GORDON, CLEARBRIDGE

LARGE AND MID-CAPS

While most investor attention in the large caps will focus on commercial execution, Borho noted that 2015 is also a big year for data catalysts in the large cap names.

The milestone of greatest interest among investors has already been announced: the Phase II RENEW data for Biogen Idec's BIIB033 anti-LINGO mAb in optic neuritis. Borho and Pinniger said, at least in the near-term, data for this compound may be more important for stock performance than Tecfidera. This is because investors have very high expectations for BIIB033 in MS, and Biogen Idec has said the optic neuritis indication will serve as proof of principle that the mAb can remyelinate nerves — the holy grail in MS. BIIB033 is in the Phase II SYNERGY trial in MS, with data expected in 2016.

"That is going to be a big event," Staijen said.

Indeed, in the 82-patient trial, BIIB033 did not meet its primary or secondary endpoints in the Phase II RENEW trial, but the company termed the results "encouraging" because they provided the first clinical evidence of the compound's effect on biological repair in the CNS by facilitating remyelination following an acute inflammatory injury.

In the trial, BIIB033 led to a 34% improvement in the primary endpoint of the recovery of optic nerve latency vs. placebo in the per-protocol population (p=0.0504). Biogen said the intent-to-treat (ITT) population showed a positive trend on the primary endpoint but did not reach statistical significance. Recovery of optic nerve latency is a measure of remyelination.

Janus Capital's Andy Acker said he's also anticipating Phase III data this year for Biogen Idec's Tysabri natalizumab in secondary progressive MS, a space that accounts for almost 25% of MS patients but where few therapeutic options exist.

Before the Kite deal, Amgen was already a pick for multiple buysiders. Smith said the big biotech's novel pipeline is underrated, and its biosimilars pipeline is even more underappreciated. This year, investors anticipate regulatory submissions for Amgen's brodalumab psoriasis candidate, which last November showed superiority vs. J&J's Stelara ustekinumab in the Phase III AMAGINE-3 trial to treat moderate to severe plaque psoriasis.

In the mid-cap space, **Receptos Inc.** was a pick for numerous investors after a strong 2014 in which the company reported positive data for its RPC1063 selective S1P1 modulator in two separate Phase II trials for MS and ulcerative colitis (UC) and raised $736.6 million in three follow-ons. Staijen noted that given the efficacy and safety profile of RPC1063 in both gastrointestinal indications and MS, Receptos is likely to be a major takeout target in 2015.

"Anybody who wants to play in the oral MS therapy space who hasn't got one already should be interested in the Receptos story," he said. He named Sanofi and **Teva Pharmaceutical Industries Ltd.** as the most likely acquirers.

Gordon said he'd be following **Portola Pharmaceuticals Inc.** this year in anticipation of Phase III data for betrixaban to treat venous thromboembolism (VTE).

SMALL CAPS

One of the more popular picks in the small cap space was **Ablynx N.V.** Muijrers said he likes the Nanobody company because "Ablynx reminds me a little bit of MorphoSys three years ago. It is a company that isn't heavily valued; they have a lot of cash, a good platform and good deals."

Bogan agreed, noting Ablynx plans to move three programs into Phase II or Phase III testing in 2015. In addition, in 2H15 Ablynx expects to report Phase IIa data for ALX-0171 to treat respiratory syncytial virus (RSV) infection. Ablynx finished the year up 29% with a market cap of $599 million and at Sept. 30 had €221.5 million ($279.7 million) in cash.

Pinniger's small cap pick for 2015 was **Alder Biopharmaceuticals Inc.**, which he thinks might have the best-in-class migraine therapy in development. In 2H15, Alder expects to announce Phase IIb data for ALD403, a mAb against calcitonin gene-related peptide (CGRP) as a preventative therapy for chronic migraine. Alder is competing with the likes of Amgen, Lilly and Pfizer, which are all also in Phase II testing with anti-CGRP mAbs.

Gordon named **CymaBay Therapeutics Inc.** as a potential under the radar name to watch in 2015. The biotech expects to report Phase IIb data for arhalofenate (MBX-102) to treat gout in 2Q15.

Muijrers' other small cap pick was **Neurocrine Biosciences Inc.** On Jan. 8, partner AbbVie said elagolix met both co-primary endpoints — a reduction in scores of non-menstrual pelvic pain and menstrual pain — in the first of two Phase III trials to treat endometriosis ($p<0.001$).

Schwarz's small cap pick was 2014 IPO **Aquinox Pharmaceuticals Inc.**, which is expected to announce Phase II data this year for AQX-1125, an oral SH2-containing inositol 5'-phosphatase (SHIP) agonist, to treat chronic obstructive pulmonary disease (COPD). bc

COMPANIES AND INSTITUTIONS MENTIONED

AbbVie Inc. (NYSE:ABBV), Chicago, Ill.
Ablynx N.V. (Euronext:ABLX), Ghent, Belgium
Achaogen Inc. (NASDAQ:AKAO), South San Francisco, Calif.
Agenus Inc. (NASDAQ:AGEN), Lexington, Mass.
Alder Biopharmaceuticals Inc. (NASDAQ:ALDR), Bothell, Wash.
Alexion Pharmaceuticals Inc. (NASDAQ:ALXN), Cheshire, Conn.
Alnylam Pharmaceuticals Inc. (NASDAQ:ALNY), Cambridge, Mass.
American Society of Hematology (ASH), Washington, D.C.
Amgen Inc. (NASDAQ:AMGN), Thousand Oaks, Calif.
Anthem Inc. (NYSE:ANTM), Indianapolis, Ind.
Aquinox Pharmaceuticals Inc. (NASDAQ:AQXP), Vancouver, B.C.
AstraZeneca plc (LSE:AZN; NYSE:AZN), London, U.K.
Avalanche Biotechnologies Inc. (NASDAQ:AAVL), Menlo Park, Calif.
Baxter International Inc. (NYSE:BAX), Deerfield, Ill.
Biogen Idec Inc. (NASDAQ:BIIB), Weston, Mass.
BioMarin Pharmaceutical Inc. (NASDAQ:BMRN), Novato, Calif.
bluebird bio Inc. (NASDAQ:BLUE), Cambridge, Mass.
Bristol-Myers Squibb Co. (NYSE:BMY), New York, N.Y.
Celgene Corp. (NASDAQ:CELG), Summit, N.J.
Celladon Corp. (NASDAQ:CLDN), San Diego, Calif.
Celldex Therapeutics Inc. (NASDAQ:CLDX), Needham, Mass.
Cellectis S.A. (Euronext:ALCLS), Paris, France
Cempra Inc. (NASDAQ:CEMP), Chapel Hill, N.C.
Conatus Pharmaceuticals Inc. (NASDAQ:CNAT), San Diego, Calif.
CVS Health Corp. (NYSE:CVS), Woonsocket, R.I.
CymaBay Therapeutics Inc. (NASDAQ:CBAY), Newark, Calif.
Eli Lilly and Co. (NYSE:LLY), Indianapolis, Ind.
Express Scripts Holding Co. (NASDAQ:ESRX), St. Louis, Mo.
Genentech Inc., South San Francisco, Calif.
Genfit S.A. (Euronext:GNFT), Loos, France
Genmab A/S (CSE:GEN; OTCBB:GMXAY), Copenhagen, Denmark
Gilead Sciences Inc. (NASDAQ:GILD), Foster City, Calif.
Incyte Corp. (NASDAQ:INCY), Wilmington, Del.
Innate Pharma S.A. (Euronext:IPH), Marseilles, France
Intercept Pharmaceuticals Inc. (NASDAQ:ICPT), New York, N.Y.
Johnson & Johnson (NYSE:JNJ), New Brunswick, N.J.
Juno Therapeutics Inc. (NASDAQ:JUNO), Seattle, Wash.
Karyopharm Therapeutics Inc. (NASDAQ:KPTI), Natick, Mass.
Kite Pharma Inc. (NASDAQ:KITE), Los Angeles, Calif.
MedImmune LLC, Gaithersburg, Md.
Merck & Co. Inc. (NYSE:MRK), Whitehouse Station, N.J.
MorphoSys AG (Xetra:MOR; Pink:MPSYF), Martinsried, Germany
Neurocrine Biosciences Inc. (NASDAQ:NBIX), San Diego, Calif.
Novartis AG (NYSE:NVS; SIX:NOVN), Basel, Switzerland
Ono Pharmaceutical Co. Ltd. (Tokyo:4528), Osaka, Japan
Paratek Pharmaceuticals Inc. (NASDAQ:PRTK), Boston, Mass.
Pfizer Inc. (NYSE:PFE), New York, N.Y.
Phenex Pharmaceuticals AG, Ludwigshafen, Germany
Portola Pharmaceuticals Inc. (NASDAQ:PTLA), South San Francisco, Calif.
ProQR Therapeutics N.V. (NASDAQ:PRQR), Leiden, the Netherlands
Prosensa Holding N.V. (NASDAQ:RNA), Leiden, the Netherlands
PTC Therapeutics Inc. (NASDAQ:PTCT), South Plainfield, N.J.
Receptos Inc. (NASDAQ:RCPT), San Diego, Calif.
Regeneron Pharmaceuticals Inc. (NASDAQ:REGN), Tarrytown, N.Y.
Relypsa Inc. (NASDAQ:RLYP), Redwood City, Calif.
Sanofi (Euronext:SAN; NYSE:SNY), Paris, France
Spark Therapeutics Inc., Philadelphia, Pa.
Synageva BioPharma Corp. (NASDAQ:GEVA), Lexington, Mass.
Tetraphase Pharmaceuticals Inc. (NASDAQ:TTPH), Watertown, Mass.
Teva Pharmaceutical Industries Ltd. (NYSE:TEVA), Petah Tikva, Israel
uniQure N.V. (NASDAQ:QURE), Amsterdam, the Netherlands
U.S. Food and Drug Administration (FDA), Silver Spring, Md.
Vertex Pharmaceuticals Inc. (NASDAQ:VRTX), Boston, Mass.
ZS Pharma Inc. (NASDAQ:ZSPH), Coppell, Texas

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