

2021 US Biotech Outlook

Now That Biotech Is Saving the World, What Comes Next?

The rather remarkable innovation engine that powers Biotech was on full display in 2020 as the sector answered the call to arms against COVID-19. That combined with a series of deals drove material outperformance for the year (NBI +28% vs. S&P500 +14% YTD) and is fueling the positive momentum into 2021. As for sustainability, we see multiple signs for optimism. These include 1) a catalyst calendar rich in important clinical readouts; 2) a generally favorable regulatory environment, with key approvals on the horizon; 3) healthy capital markets; 4) the potential for gridlock in Washington (though we certainly have to watch GA in Jan); and 5) of course, M&A, where large-caps continue to have both the cash and need to execute on deals. There are no doubt also areas of concern. The persistent lack of large-cap leadership and pockets of seemingly excessive valuations could be overhangs for 2021. Nevertheless, our favorable stance is underscored by recent buyside survey feedback, in which a large majority of respondents expect biotech to outperform the broader markets... and also see an uptick in business development deals. As usual we anticipate the 2021 J.P. Morgan Healthcare Conference to provide an early gauge on overall biotech investor sentiment with the potential for preannouncements, pipeline updates, and possible M&A.

- **For large-cap biotech**, the valuation gap relative to other healthcare subsectors and the broader market is only increasing (~11x 2020E EPS for the remaining big 3 biotech / ~13x for major pharma / ~21x healthcare overall / ~21x for the broader markets). At some point the delta might be too steep to ignore. However, the lack of large-cap leadership is a persistent source of frustration and trigger of rotation out of the group. Finding new growth drivers – either internally or externally – will be key to reversing sentiment on this front.
- **For SMID-cap biotech**, we see a humming innovation engine propelling the group with many opportunities for further upside despite some pockets of excessive valuation warranting caution. Entering 2021, with the recent flurry of M&A, we suspect larger/late-stage SMIDs with de-risked assets could come into increased focus.
- **Results from our buyside survey:** As mentioned above, 77% of survey respondents (n=69) expect biotech to outperform the broader market in 2021, driven by clinical data, favorable regulatory environment and M&A tailwinds. Of note, 79% of those surveyed expect an uptick in M&A in 2021 (compared with 56% in 2020).
- **Top-pick(s):** Kasimov – BMRN. Rama – SRPT/FOLD. Fye – ASND. Joseph – TGT. Eric – TGT.
- **Other names to consider in various investment strategies:** Growth – VRTX, JAZZ, APLS, and PTCT. Value – UTHR, TBPH, and XLRN. Event Driven – SAGE, ZYME, SWTX, ALVR, and BDTX. Launch Story – ACAD, BCRX, ZLAB, and KPTI. Turnaround – LEGN, RUBY, ANAB, and FPRX.

See page 108 for analyst certification and important disclosures.

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Biotechnology – Large Cap

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2021 US Biotech Outlook: Key Takeaways

Innovation, Vaccine Enthusiasm, Solid Fundamentals, and M&A Potential Have Biotech Well Positioned into 2021

Biotech fundamentals remain strong. . .

The innovation engine is still running at a very high level – exemplified by the rapidity of COVID-19 vaccine development

- Clinical data / innovation continues to be the #1 ranked sector tailwind for 2020 in our buyside survey (tied with increasing M&A)
- Broadly speaking, the FDA remains constructive, with 40+ new drugs/biologics approved YTD (vs. 59/46/22 in 2018/2017/2016)
- A number of high-profile/innovative biotech IPOs captured investors' attention in 2020 (e.g., Allovir, Legend, Relay, etc.)
- That said, one of the biggest fears is the pockets of excessive outperformance witnessed in 2H20

... and the value gap between Large-Cap Biotech and other HC subsectors/S&P500 may finally be too big to ignore

The lack of large-cap leadership remains a key issue but the valuation discount (~11x 2021 EPS for the remaining big 3 biotechs / 13x including emerging large caps) to healthcare overall (~21x) and the broader market (~21x for the S&P500) seems steep

- Commercial / near-commercial / platform / larger-cap SMIDs could also be the first to benefit from a sector rotation

Political headwinds could ease, at least relatively speaking

Healthcare policy discussion will continue to linger, but political/pricing headline (and fundamental) fears appear to be receding

- Legislative gridlock in 2021+ is likely to continue as an obstacle for large-scale HC reform despite bipartisan rhetoric for change
- Although 69% of buyside survey respondents expected drug pricing to be a similar or bigger overhang in 2021 vs. 2020, this is significantly up from the 52% of respondents in our 2020 survey (for 2020 vs. 2019)

Indeed, 4Q20 fund flows indicate rotation into the sector may have already started... with the factors above combined with an M&A backdrop poised to drive continued momentum in 2021

Core to the sector's fundamentals, clinical data "wins" and medical advances will always factor into enthusiasm for biotech

- While the catalyst calendar is rich heading into 2021, "wins" are important for broader investor engagement in SMIDs

M&A always has the potential to represent another tailwind

- The robust balance sheets and need for growth for large-cap biopharma could lead to an uptick in M&A activity
- Our buyside survey suggests heightened M&A is still anticipated, and increased deal activity could act as a rising tide

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2021 US Biotech Outlook: Top Picks Summary

Cory Kasimov

- **BioMarin (BMRN)** – As we move towards year-end, the BMRN story will become increasingly interesting with two value-inflecting catalysts expected in the first few weeks of 2021: one-year data from the Roctavian ph3 study potentially at the JPM Healthcare Conference in January, followed by two-year vosoritide ph3 data. We believe the probability of favorable results is high with absolute risk/reward also skewed to the upside, an unusual combination. These two candidates also have multiple clinical & regulatory catalysts throughout 2021, and valuation is compelling with the stock still trading around \$80. The bottom line is we believe BMRN checks a number boxes, including (1) solid core business, (2) being under-owned (sentiment is still fragile post CRL), (3) multiple important catalysts on the near-term horizon, (4) potential new product launches, (5) very limited/zero exposure to HC reform, and (6) minimal credit assigned to the pipeline.

Anupam Rama

- **Sarepta (SRPT)** – Phase 2 Study-102 data for lead asset SRP-9001 (micro-dystrophin gene therapy) in Duchenne Muscular Dystrophy (DMD) are expected in early 2021 and are a potentially transformational catalyst for the company. We continue to believe Study-102 has a high probability of success based on the totality of prior phase 1/2 data and what we view as achievable benchmarks for a win or homerun scenario (our detailed June 2020 slide deck, [here](#)). Recent upside in SRPT shares has shifted reward/risk but does not change our view on a positive outcome. On positive Study-102 results, we see SRPT shares trading to ~\$200-225/share+ versus down to the ~\$50-70/share range.
- **Amicus (FOLD)** – Phase 3 PROPEL data for AT-GAA (ABT200 ERT + AT2221 chaperone) in late onset Pompe Disease are anticipated in 1Q21 (our best guess is the February timeframe). Overall, we continue to view AT-GAA as having best-in-class enzyme replacement therapy (ERT) potential in Pompe Disease and see a high probability of success in the PROPEL study, based on strong pre-clinical rationale, prior phase 1/2 data, and achievable (if not beatable) benchmarks for success (see our detailed December 2020 slide deck, [here](#)). We acknowledge that the reward/risk profile of FOLD shares has shifted to be more balanced over the last ~6-8 weeks heading into PROPEL results. Still, in win/homerun scenarios, we see FOLD shares trading to the high-\$20s to mid-\$30s levels (versus down to the high-single digits to low-teens in an outright trial failure).

2021 US Biotech Outlook: Top Picks Summary, Continued

Jessica Fye

■ **Ascendis (ASND)** – Looking to 2021, we see ASND delivering on the promise of its platform heading into the launch of TransCon hGH with an attractive portfolio of clinical-stage and near-IND pipeline assets coming up behind it. We see increasing focus on the approaching US launch of TransCon hGH in mid-2021 as Ascendis transitions to a commercial-stage company, and we are optimistic about its uptake. At the same time, we see the rest of the pipeline advancing toward value-enhancing data events with TransCon PTH starting phase III (for which we expect quick enrollment and pivotal data in 2022), potential efficacy data from the phase II study of TransCon CNP in 2021, initial clinical data for ASND's first oncology candidate (TransCon TLR7/8 Agonist) by YE21, and IND/equivalent filing for TransCon IL-2 β / γ in 3Q21. Taken together, we see a compelling case for continued steady value creation from a now de-risked platform and believe the Ascendis story is still in the early innings with multiple untapped levers in the model.

Eric Joseph

■ **TG Therapeutics (TGTX)** – TGTX shares have outperformed the sector in 2020 on the heels of a number of key clinical catalysts, including positive top-line phase 3 UNITY-CLL and ULTIMATE RMS results, and solid updates from pivotal programs UNITY-CLL and -NHL at ASH 2020. Looking ahead to 2021, we see TG transitioning to a commercial-stage name with multiple regulatory updates on the horizon: i) FDA action for umbralisib in r/r MZL and r/r FL (PDUFA dates Feb. 15, 2021 and June 15, 2021) for which we forecast a combined \$450M peak sales opportunity in the US, and regulatory submissions for ii) ublituximab in RMS (~\$1.5B peak US forecast in our model), and iii) U2 in r/r and first-line CLL (~\$1.2B peak US potential). Elsewhere in the pipeline, we await longer-term results from the phase 1/2 study of U2+venetoclax and follow-up data for TG-1701, both of which should provide clarity on the development path forward. In all, we view the coming year as value defining for TGTX's heme-oncology franchise along with compelling optionality held by ublituximab in an expanding anti-CD20 RMS market, and see shares continuing to outperform our broader coverage universe.

A Quick Look Back at 2020: Views from Multiple J.P. Morgan Teams

- Performance Recap
- Multiples Analysis
- Fund Flows
- Snapshot of Binary Events

A Quick Look Back at 2020

Biotech performance ended ahead of the broader markets, but not without some turbulence along the way

Concerns around potential healthcare reform and COVID-19 weighed on the sector and led to apathetic sentiment

- While much of the year, especially early on, felt weighed down by the threat of healthcare reform, executive orders and negative presidential campaign rhetoric, concerns abated with election results implying four years of legislative gridlock (although the GA Senate runoffs could mean caution resurfaces)
- Early-spring risk-off trade (and macro sell-off) did not help either
- In 1H, the COVID-19 pandemic ignited concerns about clinical trial execution, data timelines, and commercial dynamics
- Large caps in general still left something to be desired; large-cap leadership is necessary to maintain generalist engagement with the group (no surprise, generalist interest in SMIDs was limited)

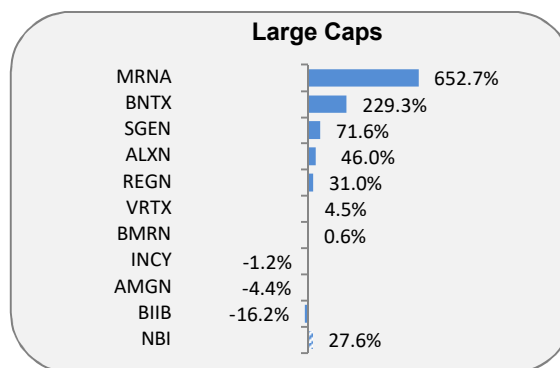
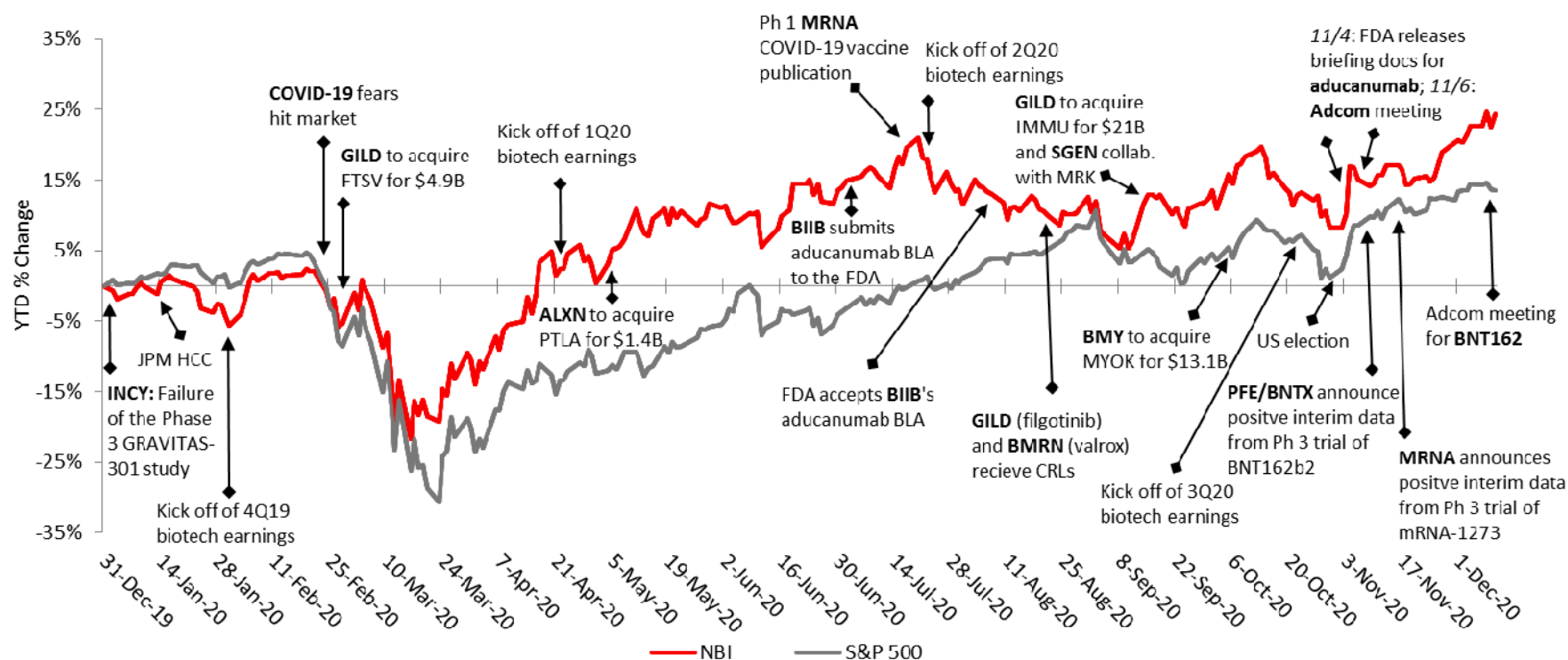
Despite COVID-19 / political headwinds, there were quite a few positive developments for biotech in 2020...

- Biotech rose to the challenge of a new pandemic and is developing both vaccines and treatments for COVID-19 at a rapid pace
- Innovation productivity continued with the approvals of a number of highly anticipated drugs/new drug classes (e.g., ALNY's Oxlumio, YMAB's Danyelza, ROG's/PTCT's Evrysdi, BMJ's/XLRN's Reblozyl, INCY's Monjuvi, RARE's Dojolvi, SGEN's Tukysa, DCPH's Qinlock, ESPR's Nexletol, etc.)
 - Many approvals further demonstrated efforts on the part of the FDA to work with sponsors on innovative therapies
- In the SMID-cap space, there were a number of clinical data wins further underscoring innovation (Apellis, Ascendis, Constellation, Denali, Mirati, Myokardia, Myovant, Karyopharm, Legend, Rocket, TG Therapeutics, etc.) highlighting the fact that investors were paid to take risk around key events
- Some M&A materialized at the Smid-cap level including a \$21B takeout of IMMU by GILD, a \$13B takeout of MYOK by BMJ, a \$6.5B takeout of MNTA by JNJ, \$4.9bn takeout of FTSV by GILD... providing strategic momentum into YE
- Capital markets activity had a record year, supporting continued funding of emerging biotechs through IPOs and secondaries
- Valuations remain depressed for large caps (ex-COVID-19 pure-plays), as the group trades at a relatively steep discount to the market (~13x 2021E EPS for big/emerging biotech vs. ~21x for the S&P)

A Quick Look Back at 2020

Biotech has outperformed the overall market since the COVID-19 outbreak

2020 YTD Performance: NBI +28% vs. S&P 500 +14%



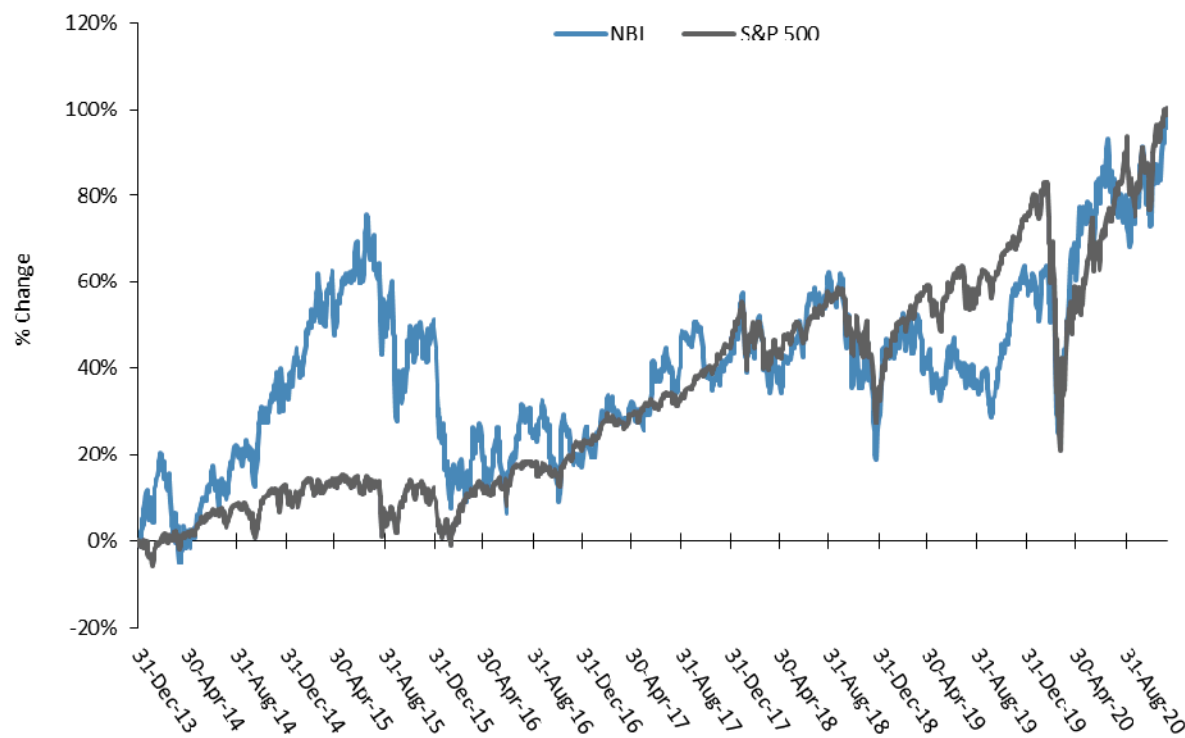
A Quick Look Back at 2020

Biotech has performed in line with the broader market in aggregate since 2014 with substantial outperformance this year

2014-2020 YTD Performance: NBI +96% vs. S&P 500 +99%

Biotech has significantly underperformed the broader markets only once (2016) over the past 7 years

- On the back of two years of underperformance, the NBI is having its strongest year since 2015 relative to the S&P 500, despite ongoing COVID-19 pandemic and political fears present for most of the year; indeed part of the sector's solutions to COVID-19 (e.g., vaccines) likely contributed to the outperformance



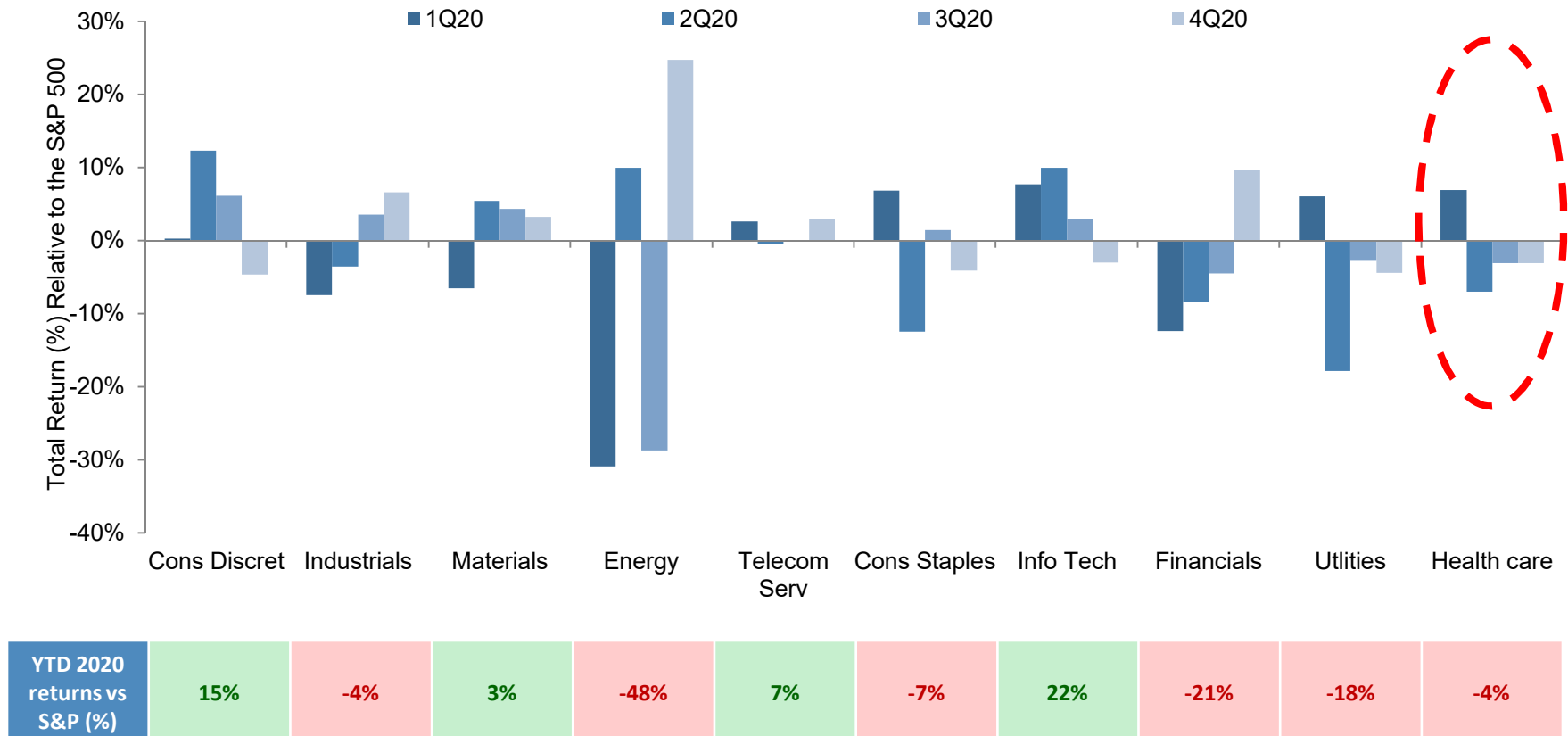
Year	NBI	S&P 500	Δ (%)
2014	34.1%	11.4%	22.7%
2015	11.4%	-0.7%	12.1%
2016	-21.7%	9.5%	-31.2%
2017	21.1%	19.4%	1.6%
2018	-9.3%	-6.2%	-3.1%
2019	24.4%	28.9%	-4.5%
2020 YTD	22.6%	13.7%	8.9%

A Quick Look Back at 2020

Healthcare has moderately trailed the S&P for the most part of 2020

While healthcare outperformed the broader markets in the first quarter of the year, it has lagged the S&P for the remainder of 2020

■ In our view, this has more to do with a strong 1Q20 and the recovery of other sectors from 1Q20 lows in subsequent quarters



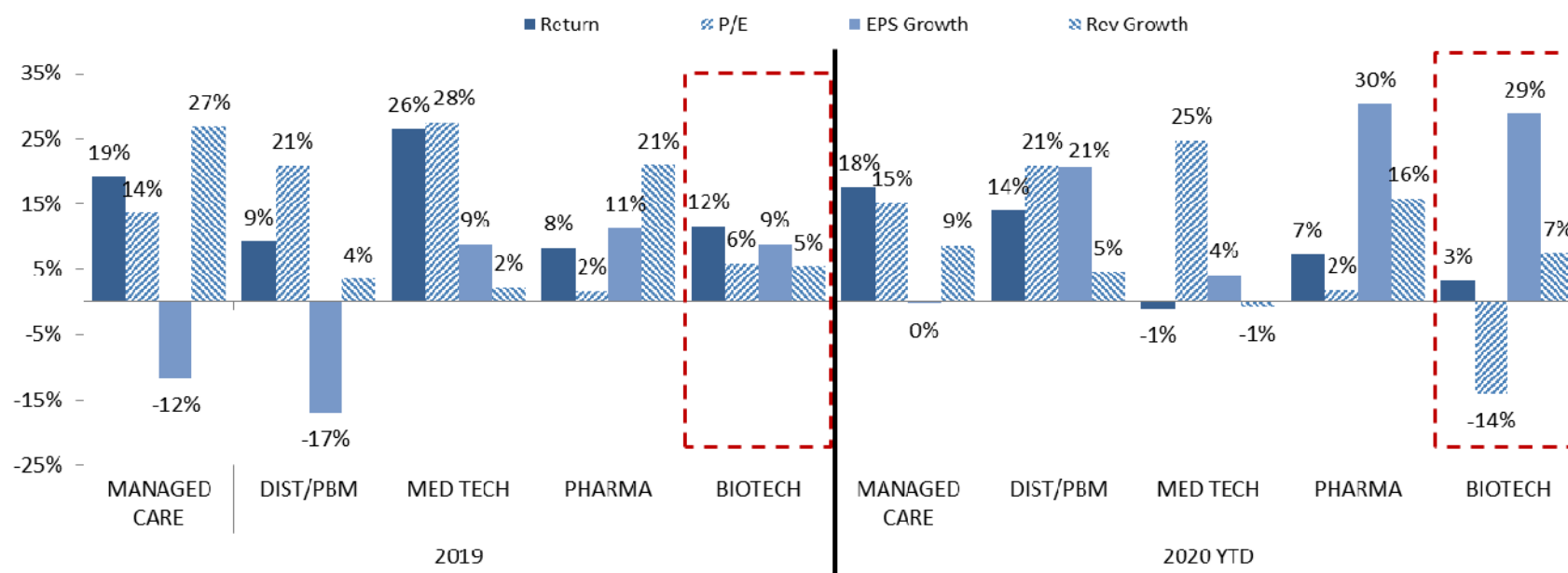
A Quick Look Back at 2020

Large-cap biotech performance was underwhelming relative to other HC sub-sectors, and low-quality EPS growth appears unsustainable

EPS growth for large-cap biotech was healthy in 2020, but quality is a question

■ Earnings leverage continued to be key for biotech in 2020, which drove acceleration on the bottom line

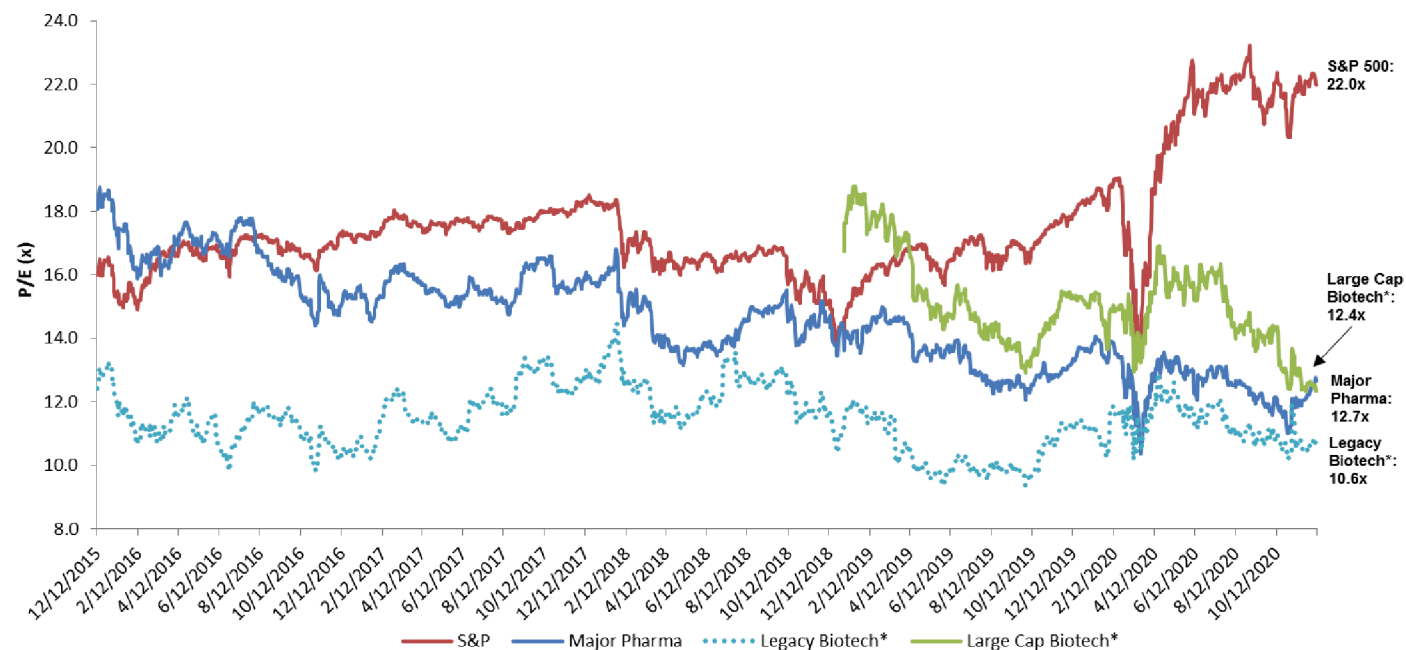
- This was boosted by aggressive share repurchases and modest revenue growth; that said, large-cap biotech (AMGN, BIIB, GILD, REGN & VRTX) revenue growth was mostly flat
- Investors were clearly uninspired by bottom-line performance in the absence of top-line growth, as biotech multiples were significantly down vs. 2019, continuing at a discount to pharma, managed care, & med-tech



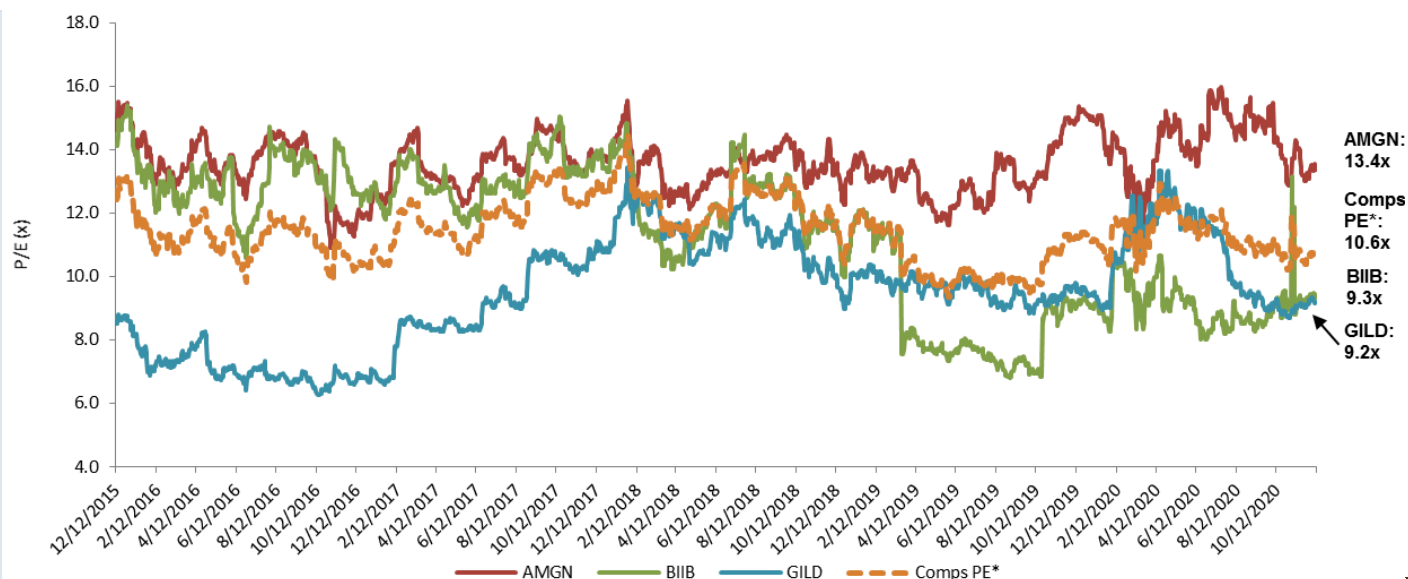
A Quick Look Back at 2020

Biotech P/E multiples continue their downward trend of the past 5 years, trading at a sizeable discount to the S&P

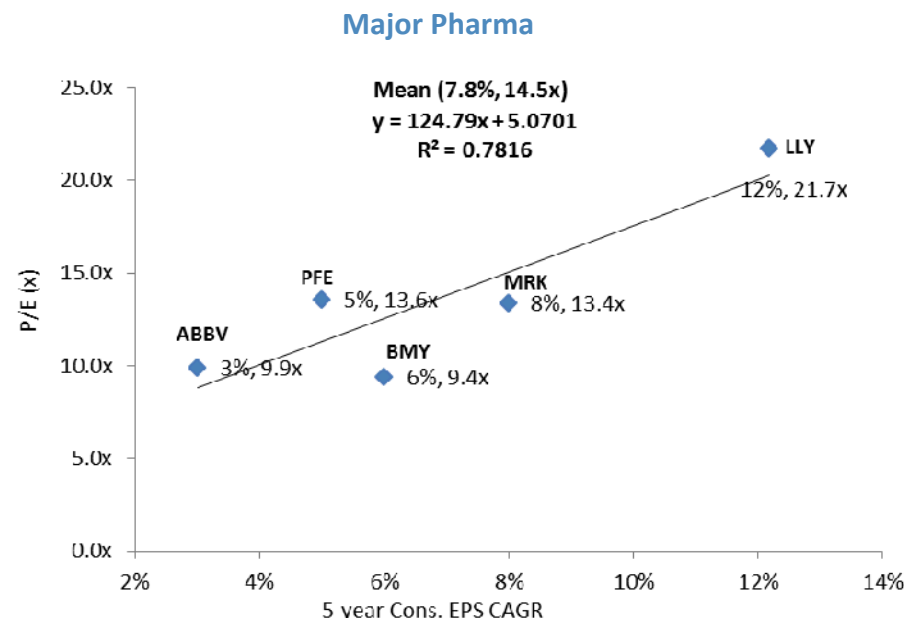
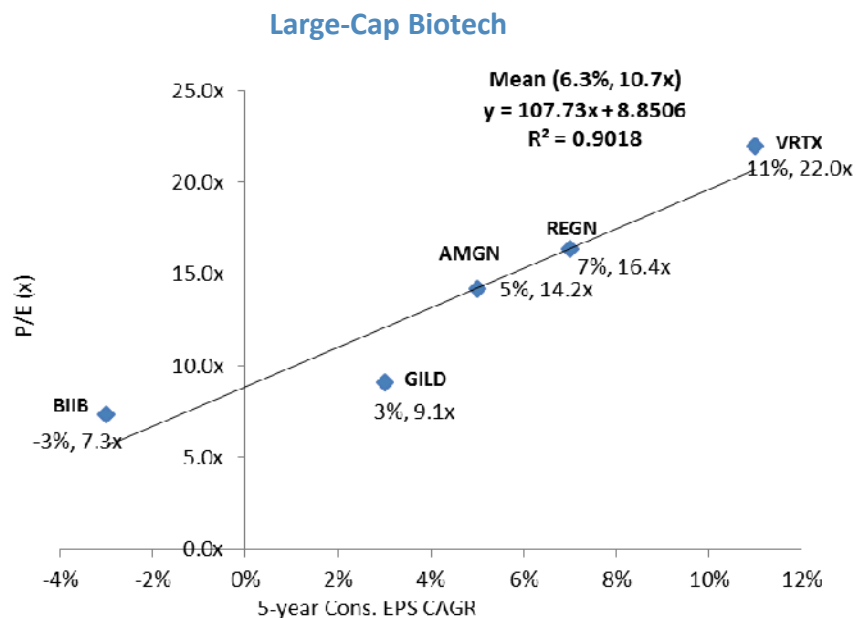
**Large-Cap
Biotech
vs.
Major Pharma
vs.
S&P 500
(last 5 years)**



**Large Caps
vs.
Biotech Peers
(last 5 years)**



Depressed multiples for BIIB & GILD potentially indicate a growing disconnect between fundamentals & price



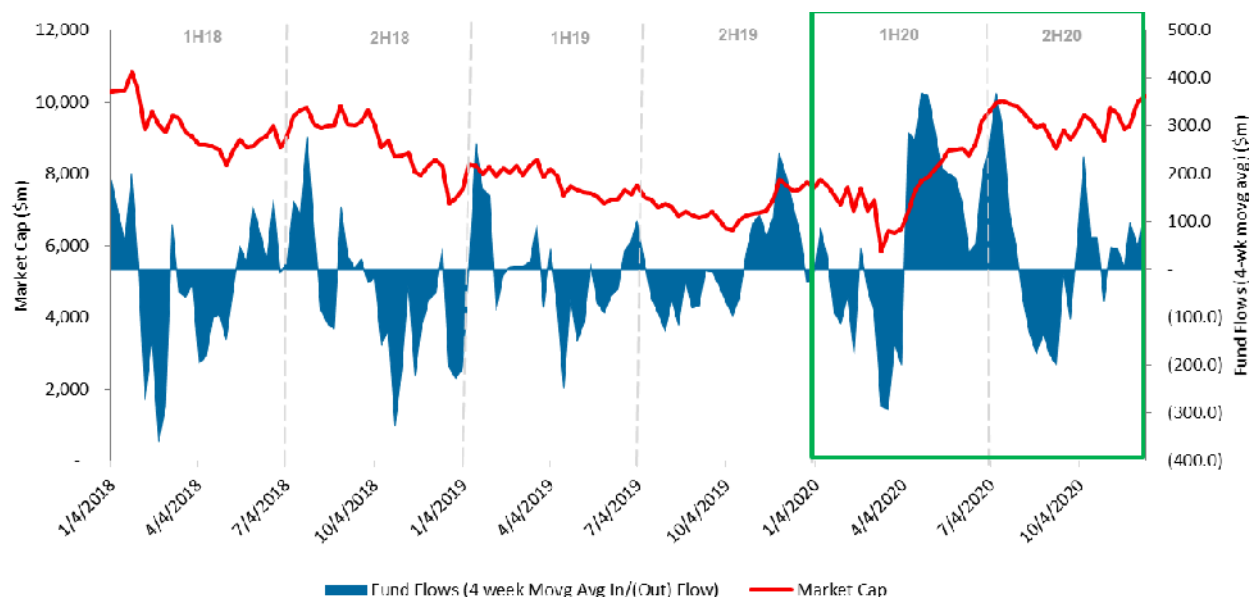
Owing to lower growth expectations than major pharma, BIIB and GILD P/E multiples are trading at a considerable discount to US major pharma and their biotech peers (AMGN, REGN & VRTX)

P/E													
Large Cap Biotech							Major Pharma						
	Company	Current P/E	Prem. / Disc. vs. bio peers	Prem. / Disc. vs. pharma peers	5-year EPS CAGR	PEG Ratio		Company	Current P/E	Prem. / Disc. vs. pharma peers	5-year EPS CAGR	PEG Ratio	
	AMGN	14.2x	3%	4%	5%	2.8x		ABBV	9.9x	-27%	3%	3.3x	
	BIIB	7.3x	-47%	-46%	-3%	-2.4x		BMY	9.4x	-31%	6%	1.6x	
	GILD	9.1x	-34%	-33%	3%	3.0x		LLY	21.7x	60%	12%	1.8x	
	REGN	16.4x	19%	21%	7%	2.3x		MRK	13.4x	-1%	8%	1.7x	
	VRTX	22.0x	59%	62%	11%	2.0x		PFE	13.6x	0%	5%	2.7x	
	Mean	13.8x			5%	1.6x		Mean	13.6x			7%	2.2x
	Median	14.2x			5%	2.3x		Median	13.4x			6%	1.8x

A Quick Look Back at 2020

A stellar year for biotech fund flows

Biotech ETF flows: though the year started off bleeding in 1Q, record net inflows were seen in 2Q, and another positive run in 4Q is leading to a huge net gain (~\$2.4B) YTD



1Q20	Week Ending	IBB (\$ M)	2Q20	Week Ending	IBB (\$ M)	3Q20	Week Ending	IBB (\$ M)	4Q20	Week Ending	IBB (\$ M)
	2-Jan-20	(164.3)		2-Apr-20	102.7		2-Jul-20	243.5		1-Oct-20	245.9
	9-Jan-20	254.4		9-Apr-20	518.7		9-Jul-20	247.8		8-Oct-20	430.3
	16-Jan-20	(165.9)		16-Apr-20	521.4		16-Jul-20	62.4		15-Oct-20	(140.9)
	23-Jan-20	(274.6)		23-Apr-20	333.5		23-Jul-20	(70.6)		22-Oct-20	(263.2)
	30-Jan-20	(268.6)		30-Apr-20	75.7		30-Jul-20	(51.6)		29-Oct-20	(289.3)
	6-Feb-20	483.4		7-May-20	221.3		6-Aug-20	(189.7)		5-Nov-20	881.4
	13-Feb-20	(648.2)		14-May-20	215.1		13-Aug-20	(222.9)		12-Nov-20	(153.6)
	20-Feb-20	608.0		21-May-20	289.4		20-Aug-20	(237.3)		19-Nov-20	(417.4)
	27-Feb-20	(601.9)		28-May-20	43.8		27-Aug-20	116.6		25-Nov-20	85.4
Q1 flows	5-Mar-20	275.7	Q2 flows	4-Jun-20	7.3	Q3 flows	3-Sep-20	(370.4)	Q4 flows	3-Dec-20	679.1
	12-Mar-20	(1,426.8)		11-Jun-20	(196.3)		10-Sep-20	(310.7)		10-Dec-20	156.3
	19-Mar-20	586.8		18-Jun-20	358.5		17-Sep-20	530.8		17-Dec-20	
	26-Mar-20	(58.0)		25-Jun-20	624.7		24-Sep-20	(264.0)		24-Dec-20	
Q1 flows		(1,400.0)	Q2 flows		3,115.8	Q3 flows		(516.3)	Q4 flows		1,214.0
Total YTD		2,413.5									

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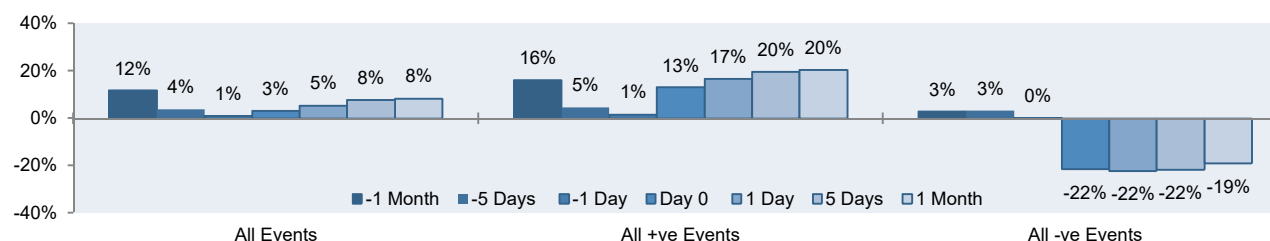
A Quick Look Back at 2020 – Snapshot of Binary Events

On average, upside following positive events was of lower magnitude than the downside after negative events in 2020, similar to 2019 & 2018

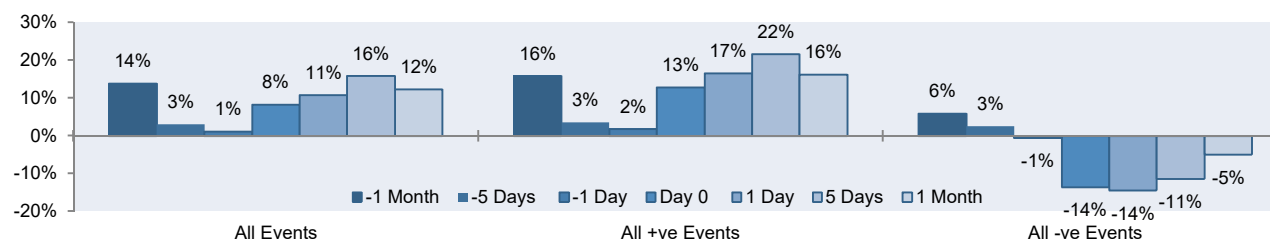
Based on an analysis of key binary events in our universe, average returns post binary events were marginally positive in 2020

- On average, the magnitude of upside post positive events was lower than the downside post negative events during the year
- Upside after positive events was slightly higher in 2H20 (vs. 1H20)
- Downside was greater on negative events in 2H20 versus 1H20

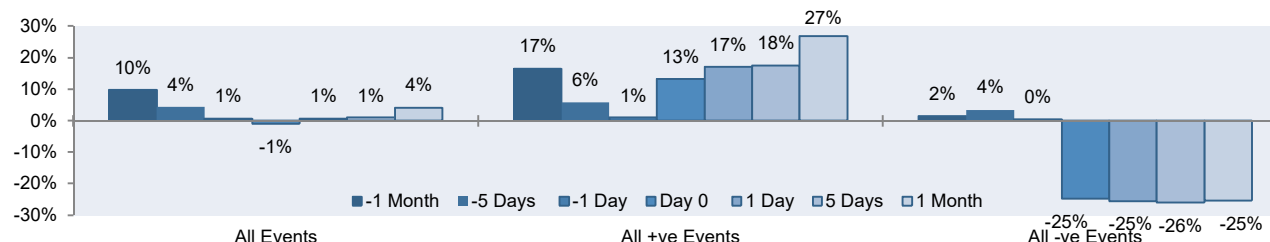
Return profiles of
Binary Events in 2020



Return profiles of
Binary Events in 1H20



Return profiles of
Binary Events in 2H20



Some of the events 1 month performances were N/A as they happened less than a month ago for 2H20 events

*Reflect Binary Events within the J.P. Morgan coverage universe. Details provided in subsequent slides

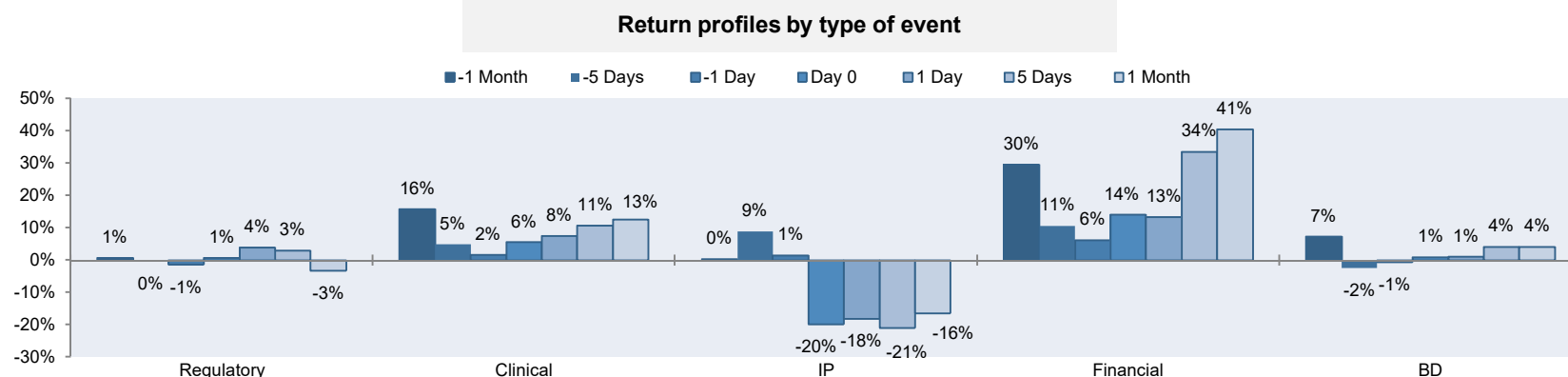
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A Quick Look Back at 2020 – Snapshot of Binary Events

On average, financial events gave maximum returns followed by clinical events; IP events corresponded with negative returns

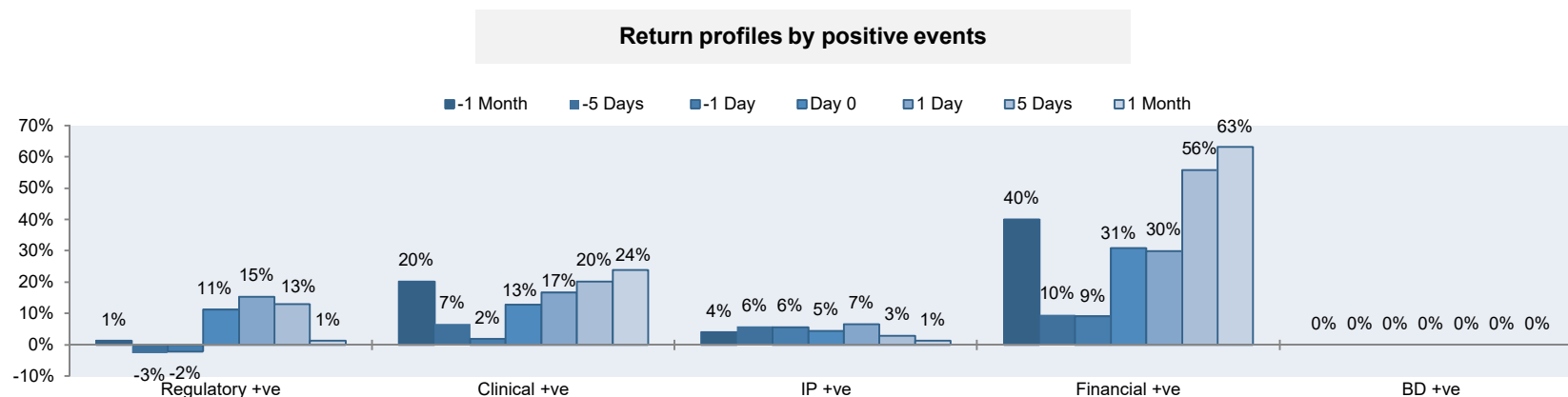
In 2020, clinical events on average returned more than the regulatory events; BD events were nearly flat, while IP events yielded negative returns

- Financial events (including quarterly earnings, funding/partnering deals) carried more upside, on average, than clinical and regulatory events



Of note, average returns post positive clinical events were higher than positive regulatory events, similar to 2019

- The magnitude of upside in 2020 on positive clinical/regulatory events was also almost similar to 2019



A Quick Look Back at 2020 – Select 1H20 Binary Events

On average, upside after positive events was slightly higher in 2H20 (vs. 1H20) and downside after negative events was lower

Ticker	Event	Type of Event	Good or Bad update?	Date of Event	% Return						
					-1 Month	-5 Days	-1 Day	Day 0	1 Day	5 Days	1 Month
INCY	Negative itacitinib data in acute GvHD	Clinical	-	01/02/20	-9%	-3%	-2%	-9%	-10%	-9%	-15%
APLS	Topline APL-2 phase 3 PEGASUS results in PNH	Clinical	+	01/07/20	26%	2%	-4%	28%	30%	34%	44%
RARE	DTX301 Ph1/2 third dose cohort data	Clinical	+	01/09/20	5%	6%	1%	-3%	20%	35%	33%
ZYME	Initial ZW49 phase I update	Clinical	+/-	01/13/20	6%	-2%	-3%	4%	-3%	9%	8%
XLRN	Phase 2 Sotatercept PULSAR	Clinical	+	01/27/20	3%	-6%	2%	50%	51%	76%	64%
VRTX	4Q19 results (included a sizeable Trikafta beat)	Financial	+	01/30/20	5%	-2%	-1%	-1%	0%	4%	-3%
BMRN	Roctavian BLA acceptance & no FDA AdCom	Regulatory	+	02/20/20	2%	2%	0%	6%	4%	0%	-19%
ESPR	Nexletol approval	Regulatory	+	02/21/20	10%	-12%	-10%	2%	-3%	-18%	-45%
UTHR	Topline Tyvaso phase III INCREASE results	Clinical	+	02/24/20	16%	3%	0%	8%	6%	-4%	-22%
ESPR	Nexlizet approval	Regulatory	+	02/26/20	16%	-19%	-5%	-8%	-11%	-2%	-34%
GILD	Gilead announces acquisition of FTSV for \$4.9B	BD	+/-	03/02/20	10%	0%	-5%	7%	10%	16%	5%
KPTI	Phase 3 BOSTON meets primary endpoint	Clinical	+	03/02/20	-4%	2%	6%	70%	51%	53%	20%
AMRN	District court decision invalidating Vascepa patents	IP	-	03/30/20	-7%	29%	3%	-71%	-63%	-65%	-44%
KNSA	Mavrilimumab COVID-19 data	Clinical	+	03/31/20	-34%	-1%	-3%	27%	23%	46%	63%
AKRO	Phase 2a BALANCED readout, 12-week PDFF	Clinical	+	03/31/20	-21%	4%	7%	23%	12%	18%	19%
XLRN	Reblozyl MDS approval and launch expansion	Regulatory	+	04/03/20	-13%	-7%	-3%	8%	6%	10%	10%
CRNX	Interim ACROBAT EDGE results	Clinical	+	04/06/20	-25%	-3%	11%	-11%	-11%	-4%	31%
URGN	Jelmyto approval in LG UTUC	Regulatory	+	04/15/20	12%	5%	-15%	5%	13%	11%	17%
ASND	Topline TransCon PTH PaTH Forward 4 week results	Clinical	+	04/19/20	28%	6%	0%	1%	1%	0%	11%
GILD	Remdesivir update in severe patients (and NIAID study statement)	Clinical	+	04/29/20	8%	0%	-2%	6%	7%	1%	-4%
ALXN	Announced acquisition of PTLA for \$1.4B	BD	-	05/05/20	16%	-6%	0%	-5%	-7%	-2%	9%
AKBA	Topline results for phase 3 INNO2VATE trial	Clinical	+	05/05/20	29%	10%	9%	38%	34%	48%	41%
AXLA	Topline AXA1125-003 results in NASH	Clinical	+	05/06/20	44%	3%	2%	16%	38%	23%	10%
BCRX	Initial BCX9930 results in PNH	Clinical	+	05/06/20	103%	18%	1%	-2%	-1%	37%	6%

A Quick Look Back at 2020 – Select 1H20 Binary Events (Cont'd)

Ticker	Event	Type of Event	Good or Bad update?	Date of Event	% Return						
					-1 Month	-5 Days	-1 Day	Day 0	1 Day	5 Days	1 Month
OYST	Topline OC-01 phase 3 ONSET-2 results in DED	Clinical	+	05/11/20	18%	13%	11%	4%	-5%	-13%	-17%
NVAX	Receives \$388M in CEPI funding	Financial	+	05/11/20	44%	36%	31%	63%	66%	132%	81%
BLUE	RFT received for Ide-cel / bb2121 in r/r MM	Regulatory	-	05/12/20	22%	1%	-6%	-2%	-5%	2%	12%
ALLO	Abstracts for initial '501 data from the ALPHA study (ASCO)	Clinical	+	05/13/20	43%	5%	-3%	-5%	29%	48%	24%
DCPH	Qinlock approval	Regulatory	+	05/15/20	5%	-12%	0%	7%	14%	23%	12%
MRNA	Updates on Phase 1 trial for COVID-19 vaccine	Clinical	+	05/18/20	42%	13%	3%	7%	10%	3%	-5%
CCXI	Topline CCX140 phase 2 LUMINA-1 results in FSGS	Clinical	-	05/18/20	17%	-1%	3%	9%	4%	12%	16%
ATRA	Updated ATA188 phase 1a results in PMS	Clinical	+	05/26/20	81%	38%	-1%	-24%	-25%	-22%	-20%
MRSN	Interim XMT-1536 phase I expansion results	Clinical	+	05/27/20	19%	8%	9%	69%	83%	102%	108%
IRWD	Discontinuation of MD-7246 for IBS	Clinical	-	05/27/20	14%	3%	-2%	-9%	-17%	-11%	-14%
ALXN	Biosimilar Soliris settlement/ IPR termination	IP	+	05/27/20	-4%	2%	3%	8%	13%	7%	7%
MYOV	Additional efficacy and safety data for Relugolix Phase 3 HERO	Clinical	+	05/29/20	0%	-6%	-3%	1%	40%	31%	69%
REPL	Updated RP1 phase 1/2 data in solid tumors	Clinical	+	06/03/20	23%	-2%	1%	1%	18%	1%	11%
AGIO	Mitapivat proof of concept updates in SCD (ph 1) and thalassemia (ph 2)	Clinical	+	06/12/20	15%	2%	-4%	-7%	-5%	-5%	4%
JAZZ	Zepzelca accelerated approval	Regulatory	+	06/15/20	-1%	-1%	6%	1%	0%	2%	-1%
BIIB	Federal ruling that Tecfidera '514 patent is invalid	IP	-	06/18/20	-11%	-5%	0%	-8%	-4%	-7%	0%
KPTI	Xpovio approval in DLBCL	Regulatory	+	06/22/20	8%	14%	2%	0%	4%	-3%	0%
MYOV	Phase 3 SPIRIT 1 in endometriosis	Clinical	+	06/23/20	37%	3%	4%	14%	17%	11%	-3%
AKRO	Phase 2a BALANCED 16-week histology readout	Clinical	+	06/30/20	-2%	-5%	9%	34%	33%	49%	41%
All Events					14%	3%	1%	8%	11%	16%	12%
All +ve Events					16%	3%	2%	13%	17%	22%	16%
All -ve Events					6%	3%	-1%	-14%	-14%	-11%	-5%

A Quick Look Back at 2020 – Select 2H20 Binary Events

On average, upside after positive events was slightly higher in 2H20 vs. 1H20 and downside after negative events was higher

Ticker	Event	Type of Event	Good or Bad update?	Date of Event	% Return						
					-1 Month	-5 Days	-1 Day	Day 0	1 Day	5 Days	1 Month
AMGN	Enbrel IP: Appeal Decision	IP	+	07/01/20	12%	10%	8%	1%	0%	-1%	-4%
OBSV	Topline results for PRIMROSE 1 and 2	Clinical	-	07/06/20	32%	3%	3%	-47%	-52%	-55%	-59%
NVAX	Receives \$1.6B in OWS funding	Financial	+	07/07/20	72%	-5%	-3%	32%	24%	31%	111%
MRNA	Phase 1 publication for COVID-19 vaccine	Clinical	+	07/14/20	21%	23%	5%	7%	9%	8%	-8%
TCDA	FDA letter foreshadowing CRL for veverimer	Regulatory	-	07/15/20	-3%	-3%	1%	-40%	-37%	-44%	-46%
ACAD	Ph3 data for Nuplazid in MDD	Clinical	-	07/20/20	6%	-1%	1%	-19%	-22%	-21%	-29%
JAZZ	Xywav approval	Regulatory	+	07/22/20	-7%	-2%	-2%	4%	7%	8%	17%
AXLA	Topline AXA1665-002 results in OHE	Clinical	+	08/05/20	-1%	16%	5%	2%	0%	5%	-15%
PTCT	Evrysdi FDA approval for SMA	Regulatory	+	08/07/20	-13%	3%	2%	0%	0%	2%	0%
PASG	PBGM01 (GM1) Clinical Hold Announced	Regulatory	-	08/13/20	-35%	-2%	-7%	4%	-4%	-7%	5%
BMRN	Roctavian CRL	Regulatory	-	08/19/20	-8%	1%	-1%	-35%	-37%	-37%	-33%
AMRN	Oral arguments at US Court of Appeals	IP	-	09/02/20	12%	9%	-6%	-31%	-37%	-40%	-41%
AKBA	Topline results for phase 3 PRO2TECT trial	Clinical	-	09/03/20	-10%	-1%	1%	-74%	-73%	-74%	-75%
ITCI	Topline Caplyta phase III Study 402 results	Clinical	+	09/09/20	-12%	5%	2%	73%	68%	65%	47%
GILD	Gilead announces acquisition of IMMU for \$21B	BD	+/-	09/14/20	-4%	-1%	3%	1%	0%	-1%	-2%
ASND	Topline TransCon PTH PaTH Forward 6 mo OLE results	Clinical	+	09/28/20	9%	8%	-1%	0%	2%	6%	6%
REGN	Initial clinical data for COVID-19 antibody cocktail	Clinical	+/-	09/29/20	-5%	0%	0%	-2%	-1%	1%	-3%
MYOV	Phase 3 HERO CRF survival readout	Clinical	-	09/29/20	6%	-6%	2%	-26%	-33%	-31%	-31%
IRWD	Discontinuation of IW-3718 for refractory GERD	Clinical	-	09/29/20	-6%	-2%	1%	-1%	-6%	-2%	0%
BCRX	Updated BCX9930 results in PNH	Clinical	+	09/30/20	-3%	2%	4%	-10%	-8%	-9%	-3%
SLDB	Phase 1/2 IGNITE-DMD Clinical Hold Lift	Regulatory	+	10/01/20	-15%	-6%	-1%	70%	106%	102%	61%
AMGN	Topline Ph2 Data for sotorasib/KRASG12C in NSCLC	Clinical	+	10/05/20	3%	4%	4%	-1%	1%	-6%	-9%
YMAB	RTF for BLA for Omplasty in CNS/LM from NB	Regulatory	-	10/05/20	8%	9%	7%	-9%	-2%	-6%	17%
AMGN	Omecamtiv mecarbil Ph3 results	Clinical	-	10/08/20	4%	1%	2%	-7%	-8%	-8%	-10%
JAZZ	Topline Xywav phase III results in IH	Clinical	+	10/08/20	9%	1%	2%	8%	8%	6%	4%
ANAB	Phase 2 GALLOP results	Clinical	+	10/13/20	16%	4%	-1%	6%	22%	53%	76%

A Quick Look Back at 2020 – Select 2H20 Binary Events (Cont'd)

Ticker	Event	Type of Event	Good or Bad update?	Date of Event	% Return						
					-1 Month	-5 Days	-1 Day	Day 0	1 Day	5 Days	1 Month
VRTX	VX-814 in AATD discontinued	Clinical	-	10/14/20	3%	2%	-2%	-21%	-20%	-22%	-17%
REPL	Initial RP2 phase 1 data in solid tumors	Clinical	+	10/14/20	1%	-2%	0%	47%	66%	72%	86%
SAGE	Phase 3 open label zuranolone SHORELINE data	Clinical	+	10/15/20	11%	-3%	0%	4%	8%	5%	24%
APTX	Topline NYX-783 phase II results in PTSD	Clinical	+/-	10/19/20	5%	-6%	-2%	17%	7%	-19%	-13%
REPL	Updated RP1 phase 1/2 data in solid tumors	Clinical	+	10/20/20	71%	72%	1%	-2%	2%	6%	18%
MRTX	Durability data for MRTX849 in 2L+ NSCLC	Clinical	+	10/25/20	13%	-9%	0%	9%	16%	20%	25%
CRNX	Topline ACROBAT EDGE and EVOLVE results	Clinical	+	10/26/20	2%	-18%	-5%	-10%	-6%	-13%	-7%
CCXI	Topline avacopan phase 2b AURORA results in HS	Clinical	+/-	10/28/20	-2%	0%	0%	-5%	-8%	2%	7%
TCDA	Update following Type A meeting	Regulatory	-	10/29/20	-7%	-6%	4%	-47%	-32%	-14%	-10%
BIIB	FDA released aducanumab briefing docs ahead of AdCom	Regulatory	+	11/04/20	-11%	-3%	-1%	44%	33%	-4%	-1%
GBT	3Q results (which included a sizable Oxbryta miss)	Financial	-	11/05/20	-2%	13%	-3%	-37%	-36%	-33%	-27%
BIIB	Aducanumab AdCom panel	Regulatory	-	11/06/20	17%	30%	0%	-28%	-28%	-24%	-25%
BNTX	Phase 2/3 interim analyses for COVID-19 vaccine	Clinical	+	11/09/20	7%	8%	0%	14%	23%	15%	30%
ARNA	Topline etrasimod phase II ADVISE results	Clinical	-	11/09/20	6%	6%	1%	-20%	-23%	-24%	-21%
ATRA	Positive interim analysis for tab-cel in EBV+ PTLTD	Clinical	+	11/09/20	0%	14%	7%	8%	9%	36%	60%
DNLI	Topline DNL310 phase I results	Clinical	+	11/10/20	34%	28%	3%	8%	19%	14%	NA
MRNA	Interim results from the Phase 3 COVID-19 vaccine	Clinical	+	11/16/20	18%	23%	2%	4%	-1%	9%	75%
LXRX	SOLOIST and SCORED results	Clinical	+	11/17/20	-12%	11%	2%	40%	24%	13%	NA
FPRX	Topline results from phase 2 FIGHT trial in gastric/GEJ cancer	Clinical	+	11/20/20	213%	-17%	-2%	-2%	4%	7%	NA
ALNY	U.S. Oxlumio Approval in PH1	Regulatory	+	11/23/20	-7%	-6%	-1%	2%	2%	6%	NA
YMAB	U.S. Approval of Danyelza in high-risk R/R NB	Regulatory	+	11/25/20	6%	-2%	-3%	10%	16%	16%	NA
AGIO	Topline mitapivat phase 3 ACTIVATE results in PKD	Clinical	+	12/01/20	16%	5%	4%	0%	0%	-5%	NA
JAZZ	ATLANTIS results	Clinical	-	12/03/20	-2%	2%	1%	4%	4%	1%	NA
BCRX	Orladeyo approval	Regulatory	+	12/03/20	30%	8%	2%	19%	41%	41%	NA
ALLO	Phase 1 ALLO-715 update at ASH	Clinical	-	12/05/20	8%	7%	4%	-14%	-12%	NA	NA
All Events					10%	4%	1%	-1%	1%	1%	4%
All +ve Events					17%	6%	1%	13%	17%	18%	27%
All -ve Events					2%	4%	0%	-25%	-25%	-26%	-25%

Looking Ahead to 2021: Views from Multiple J.P. Morgan Teams

- JPM US Equity Strategy View
- JPM Trading Desk Perspective
- JPM Biotech Team Overview

Looking Ahead to 2021 – JPM US Equity Strategy View

J.P. Morgan US Equity Strategy Team likes Healthcare from a growth, value, as well as a quality perspective

In its 2021 outlook for US Equity Markets ([here](#)), the JPM US Equity Strategy Team said it expects a pent-up recovery to drive another year of healthy 7% earnings growth for Healthcare, highlighting that the sector trades at only 17x based on 2021 consensus estimates vs. 24x for the S&P 500

- “At the sector level, the central theme next year will likely be COVID-19 recovery with easing lockdowns releasing pent-up demand and reflation. The largest beneficiaries should be stocks at the epicenter of the 2020 pandemic: Consumer Discretionary (in particular, Consumer Services such as Leisure / Gaming / Hotels, Off-line Retail), Financials (Banks, Consumer Finance), and Energy. **We also like Healthcare for its strong defensive growth, below market valuation, and reducing headline risk with an expected gridlock in Congress.**”
- “Healthcare checks off growth, value, and quality characteristics and is a steady performer due to strong secular tailwinds. With significant political overhang (e.g., Blue Wave) waning, Healthcare should be well positioned to benefit from economic normalization, growing M&A activity and steady sector fundamentals.”

	NI Margin (Avg Last 4Qs)				
	2019	2020	Delta	2021	Delta
Energy	5.2%	-0.7%	-5.9%	2.5%	3.2%
Materials	9.4%	8.7%	-0.7%	10.0%	1.3%
Industrials	9.5%	5.6%	-3.9%	8.9%	3.3%
Discretionary	7.7%	4.5%	-3.2%	6.5%	2.0%
Staples	6.9%	6.8%	-0.1%	6.9%	0.1%
HealthCare	10.1%	10.1%	0.0%	10.5%	0.4%
Financials	19.4%	14.0%	-5.3%	16.5%	2.4%
Technology	22.1%	21.9%	-0.2%	22.6%	0.7%
Communication Svc	15.0%	14.0%	-1.0%	14.8%	0.8%
Utilities	9.4%	12.0%	2.6%	12.6%	0.6%
Real Estate	21.1%	14.8%	-6.2%	13.7%	-1.1%
S&P 500	11.7%	10.0%	-1.72%	11.3%	1.27%
Ex-Energy	12.3%	10.7%	-1.67%	11.9%	1.20%

	S&P 500 Earnings				2020 Earnings Contribution		
	2019	2020E	2021E	% of Total	Net Inc. Δ (\$)	% Chg	to Earnings Growth
Energy	\$53	(\$5)	\$20	1.5%	\$25	-538.4%	10%
Materials	\$27	\$21	\$26	2.0%	\$5	24.3%	2%
Industrials	\$127	\$64	\$111	8.3%	\$48	75.2%	19%
Discretionary	\$117	\$72	\$116	8.6%	\$44	61.5%	18%
Staples	\$107	\$109	\$114	8.4%	\$5	4.7%	2%
HealthCare	\$216	\$234	\$261	19.4%	\$27	11.3%	11%
Financials	\$241	\$173	\$206	15.3%	\$33	19.3%	13%
Technology	\$282	\$292	\$328	24.3%	\$35	12.0%	14%
Communication Svc	\$145	\$139	\$163	12.1%	\$25	17.7%	10%
Utilities	\$2	\$2	\$2	0.2%	\$0	11.1%	0%
Real Estate	\$22	\$15	\$15	1.1%	(\$0)	-2.2%	0%
S&P 500	\$1,317	\$1,101	\$1,348	100%	\$247	22.4%	100%

Looking Ahead to 2021 – JPM US Equity Strategy View

Healthcare is expected to contribute 17% of S&P500's revenue growth, the second highest for any sector...

	S&P 500 Sales				2020 Sales Contribution		
	2019	2020E	2021E	% of Total	Sales Δ (\$)	% Chg	Contrib. to Sales Growth
Energy	\$1,021	\$675	\$799	6.4%	\$124	18.3%	13%
Materials	\$358	\$315	\$339	2.7%	\$24	7.7%	3%
Industrials	\$1,368	\$1,163	\$1,280	10.2%	\$117	10.0%	12%
Discretionary	\$1,426	\$1,438	\$1,642	13.1%	\$204	14.2%	22%
Staples	\$1,560	\$1,610	\$1,652	13.2%	\$42	2.6%	4%
HealthCare	\$2,152	\$2,333	\$2,498	20.0%	\$165	7.1%	17%
Financials	\$1,580	\$1,487	\$1,523	12.2%	\$35	2.4%	4%
Technology	\$1,275	\$1,335	\$1,447	11.6%	\$112	8.4%	12%
Communication Svc	\$968	\$991	\$1,104	8.8%	\$113	11.4%	12%
Utilities	\$220	\$212	\$220	1.8%	\$8	3.7%	1%
Real Estate	\$109	\$106	\$112	0.9%	\$6	5.6%	1%
S&P 500	\$11,928	\$11,560	\$12,504	100%	\$944	8.2%	100%

... while Biotech is expected to deliver 8% revenue and earnings growth

S&P 500 Current Constituents

	Sales Growth						NI Margin (Avg Last 4Qs)					Earnings Growth						Valuation — PE	
	2021/1C	2021/2C	2021/3C	2021/4C	2020	2021	2019	2020	Delta	2021	Delta	2021/1C	2021/2C	2021/3C	2021/4C	2020	2021	2020	2021
Energy	-15%	61%	27%	22%	-33.9%	18.3%	5.2%	-0.7%	-5.9%	2.5%	3.2%	-65%	-141%	-650%	7630%	-109%	-538%	-166.9x	38.1x
Materials	3%	16%	8%	5%	-12.1%	7.7%	9.4%	8.7%	-0.7%	10.0%	1.3%	19%	44%	19%	17%	-20%	24%	26.4x	21.3x
Industrials	-4%	20%	14%	12%	-15.0%	10.0%	9.5%	5.6%	-3.9%	8.9%	3.3%	-2%	335%	77%	71%	-50%	75%	41.7x	23.8x
Discretionary	12%	23%	10%	12%	0.8%	14.2%	7.7%	4.5%	-3.2%	6.5%	2.0%	81%	233%	11%	52%	-39%	62%	52.1x	32.2x
Staples	0%	4%	3%	3%	3.3%	2.6%	6.9%	6.8%	-0.1%	6.9%	0.1%	1%	9%	1%	9%	2%	5%	22.1x	21.1x
HealthCare	8%	11%	5%	5%	8.4%	7.1%	10.1%	10.1%	0.0%	10.5%	0.4%	15%	7%	7%	17%	8%	11%	18.0x	16.2x
Financials	25%	-9%	-5%	5%	-5.9%	2.4%	19.4%	14.0%	-5.3%	16.5%	2.4%	51%	64%	-10%	2%	-28%	19%	16.7x	14.0x
Technology	10%	8%	9%	7%	4.7%	8.4%	22.1%	21.9%	-0.2%	22.6%	0.7%	10%	12%	10%	15%	4%	12%	30.5x	27.2x
Communication Svc	9%	16%	12%	9%	2.4%	11.4%	15.0%	14.0%	-1.0%	14.8%	0.8%	11%	29%	10%	21%	-5%	18%	27.7x	23.5x
Utilities	6%	6%	4%	0%	-3.3%	3.7%	9.4%	12.0%	2.6%	12.6%	0.6%	0%	8%	16%	29%	11%	11%	17.7x	16.0x
Real Estate	-2%	9%	7%	9%	-2.7%	5.6%	21.1%	14.8%	-6.2%	13.7%	-1.1%	-37%	-3%	39%	19%	-31%	-2%	50.9x	52.1x
S&P 500	6.3%	11.6%	7.1%	7.8%	-3.1%	8.2%	11.7%	10.0%	-1.72%	11.3%	1.27%	16.4%	45.5%	11.6%	21.9%	-16%	22%	27.3x	22.3x
Ex-Energy	8.0%	9.3%	6.0%	7.0%	-0.2%	7.5%	12.3%	10.7%	-1.67%	11.9%	1.20%	18.6%	37.2%	9.1%	19.5%	-13%	20%	26.5x	22.0x
Health Care Equipr	20%	27%	7%	4%	0.1%	13.6%	20.1%	18.5%	-1.6%	21.3%	2.9%	49%	89%	13%	8%	-8%	31%	34.8x	26.5x
Health Care Providr	6%	9%	4%	5%	9.1%	5.8%	3.5%	3.5%	0.1%	3.5%	0.0%	13%	-26%	18%	48%	11%	7%	14.6x	13.7x
Health Care Technr	0%	6%	5%	5%	-3.3%	3.9%	15.1%	15.9%	0.8%	16.7%	0.7%	2%	19%	9%	6%	2%	9%	25.8x	23.8x
Biotechnology	17%	12%	1%	3%	16.8%	7.9%	39.4%	38.0%	-1.4%	38.1%	0.1%	15%	13%	-2%	10%	12%	8%	11.9x	11.0x
Pharmaceuticals	6%	16%	9%	7%	6.2%	9.2%	28.5%	29.5%	1.0%	29.1%	-0.4%	1%	12%	5%	14%	10%	8%	15.3x	14.2x

Looking Ahead to 2021 – The Trading Perspective

Perspective from our desk... what they're seeing and what the vibe is into 2021

- Thoughts from the J.P. Morgan Healthcare Trading Desk ([conference call commentary](#))

Looking Ahead to 2021 – Thoughts from the JPM Biotech Team

The promise of breakthrough COVID-19 vaccines and therapeutics buoyed the sector throughout the turbulence of 2020, and we expect that favorable sentiment to carry forward into 2021 driven by solid sector fundamentals

With the world poised to return to a “new normal,” we see a relatively favorable setup for the sector in 2021 given attractive relative valuation amongst the large caps, a fairly robust catalyst calendar, and an M&A tailwind

- We continue to see solid sector fundamentals, and in 2021 expect: (1) important clinical wins (underscoring strong innovation); (2) accessible capital markets; (3) persistent need for M&A; and (4) a number of strong product launches
- We expect the 2021 J.P. Morgan Healthcare Conference in January to represent an early gauge on overall biotech investor sentiment and an opportunity for a fresh look at new ideas
 - Despite being virtual, our sense is that companies and investors are treating the conference similarly to prior years

While we expect bipartisan support for healthcare reform to continue, sweeping change to the healthcare system is unlikely with some caveats...

- Looking to 2021, we believe we may be past the political sentiment nadir given: (1) clear challenges of implementing anything close to “Medicare for All”; and (2) an overall lack of alignment on specific reform policies
- That said, we have to acknowledge that sentiment has the potential to change on a dime pending Senate election results from Georgia, which could allow the Democrats to take full control of Congress

Biotech looks poised to benefit from potential market rotation

- We continue to believe that the relative valuation gap remains too big to ignore (large-cap biotech trades at a ~39% discount to S&P500 on 2021e EPS), despite the nagging lack of large-cap leadership with this group

Looking Ahead to 2021 – Historical Returns at the JPM HC Conference

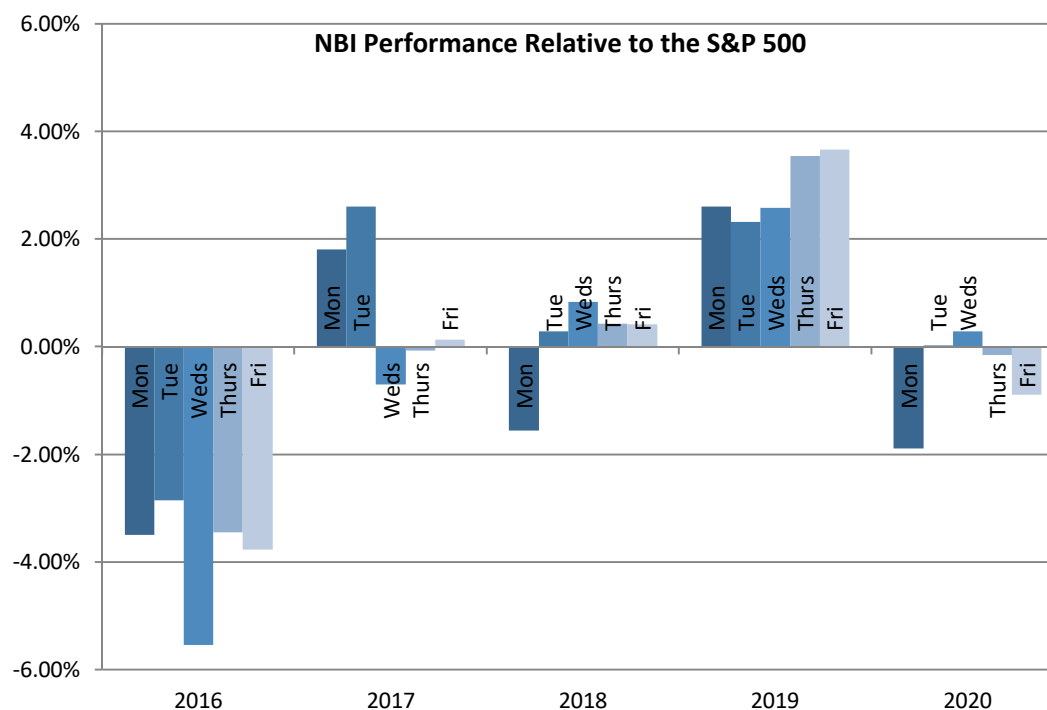
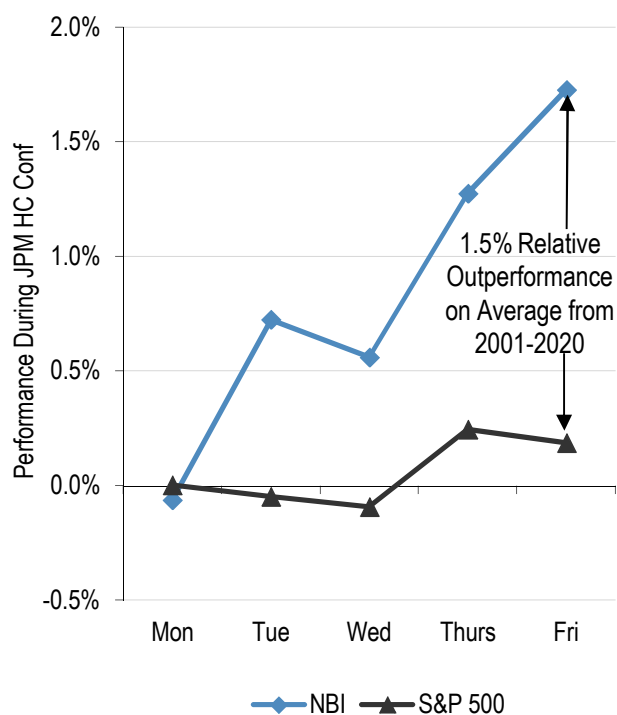
Biotech has historically outperformed the broader market during the J.P. Morgan Healthcare Conference... 2020 was a rare exception

We evaluated NBI performance vs the S&P 500 during the JPM Healthcare Conference over the past 20 years

- ~80% of the time (for all but 4 years), the NBI has outperformed the S&P 500 during the week of the J.P. Morgan HC Conference
- The NBI outperformed the S&P 500 by ~1.5%, on average, during the week of the conference over this two-decade span; last year's conference was one of the anomalies with the sector underperforming the broader markets by a marginal ~1%

Many will be looking to the 2021 conference as an early gauge on the overall sector for 2021

- As usual, key levers to monitor include potential M&A, clinical/regulatory pipeline updates, the catalyst setup for the year ahead, and overall engagement



Looking Ahead to 2021 – the Large Caps

What will it likely take for large-cap biotech companies to work – or not work – in 2021?

AMGN

- **It works if...** sotorasib demonstrates differentiation, teze data support broad utilization, BMY's full TYK2 data disappoint, new product cycle gains greater traction (Aimovig and Otezla, in particular), biosimilar impact is more modest than expected, proprietary biosimilar momentum continues and/or AMGN executes on well received M&A transactions to add growth drivers
- **It doesn't work if...** late-stage pipeline is undifferentiated (sotorasib/teze in particular), Aimovig loses share to new entrants (such as oral CGRP), Otezla growth disappoints, biosimilar risk intensifies (on Neulasta and/or other legacy products), proprietary biosimilar momentum fades, and/or clinical catalysts don't materialize

GILD

- **It works if...** more clarity is gained in the company's strategic direction and the commercial opportunities for assets associated with IMMU & FTSV M&A transactions, Biktarvy/HIV sales continue to beat estimates, remdesivir appears more durable than currently anticipated, the Yescarta launch gains traction and the 2L DLBCL study reads out positively, or additional pipeline candidates emerge either internally and/or through additional M&A
- **It doesn't work if...** The status quo is maintained, assets acquired through M&A disappoint, Biktarvy/HIV performance lags, there is unfavorable news on the Biktarvy IP front, Yescarta growth continues to be underwhelming, and/or further M&A does not materialize

MRNA

- **It works if...** mRNA-1273 revenues appear durable, competitor updates disappoint, or additional proprietary assets emerge that many have sizable near-term opportunities
- **It doesn't work if...** vaccine uptake is underwhelming, competitor COVID-19 vaccines Phase 3 trials show comparable benefit or significant global uptake, or the COVID-19 pandemic is contained faster than expected calling long-term mRNA-1273 revenues into question

VRTX

- **It works if...** Trikafta continues to impress and drive sales for the overall CF portfolio, pipeline assets deliver promising data (AATD rises from the dead with positive VX-864 data), and the company executes on quality BD
- **It doesn't work...** Trikafta growth is underwhelming, additional pipeline failures, aggressive spending limits bottom-line growth, and/or competitive concerns in CF re-intensify. Further, with the story begging "what's next for Vertex beyond CF?" if the pipeline fails to deliver, shares could be range-bound in the near term

Looking Ahead to 2021 – the Large Caps (continued)

What will it likely take for large-cap biotech to work – or not work – in 2021?

REGN

- **It works if...** Eylea is able to grow relative to current expectations and competitor updates underwhelm, Dupi's strong launch in AD / other allergic indications continues, there are well-received clinical updates from the IO pipeline, REGN-CoV2 (antibody cocktail) revenues appear more durable, and/or we get additional pipeline surprises
- **It doesn't work if...** Dupi fails to exceed commercial expectations or add'l competitive overhangs emerge, Eylea growth stalls or looming LOE becomes a bigger headwind, or the IO pipeline disappoints

BIIB

- **It works if...** Quite simply if aducanumab is approved (3/7/21 PDUFA) without the need for an additional Phase 3 pre-approval; of lesser near-term significance, BIIB can benefit from zuranolone wins in the series of Phase 3s in 2021 (from the SAGE collaboration) and the impact of Spinraza competition is less than feared
- **It doesn't work if...** Aducanumab isn't approved, zuranolone falls short, generic MS puts a greater dent on the top line than currently anticipated, and/or Spinraza competitive updates negatively impact the future outlook of the product

SGEN

- **It works if...** Padcev and Tukysa launches exceed expectations, TV is approved and early launch metrics prove favorable, we get additional pipeline surprises, and/or the move to multi-product story continues to feed recent momentum (and strategic optionality)
- **It doesn't work if...** SGEN's commercial franchises disappoint (Padcev and/or Tukysa in particular), there are any regulatory setbacks for TV, or the perceived strategic interest for SGEN dissipates

BNTX

- **It works if...** BNT162b2 revenues appear likely to be durable, competitor updates disappoint, or further assets (oncology or infectious disease) emerge that may have sizable near-term opportunities
- **It doesn't work if...** vaccine uptake is underwhelming, competitor COVID-19 vaccines Phase 3 trials show comparable benefit or significant global uptake, or the COVID-19 pandemic is contained faster than expected calling long-term BNT162b2 revenues into question

Looking Ahead to 2021 – the Large Caps (continued)

What will it likely take for large-cap biotech to work – or not work – in 2021?

INCY

- **It works if...** Jakafi growth continues, Monjuvi launch impresses, management effectively manages cost structure, and the company executes on its broad pipeline (topical rux in vitiligo, in particular)
- **It doesn't work if...** Jakafi commercial trends start to slow, Monjuvi launch is underwhelming, the company fails to produce encouraging results in multiple ongoing programs, or investors resist re-engaging for what some consider to be more incremental opportunities

NVCR

- **It works if...** Optune demonstrates clinical activity outside of the approved indications (starting with the Ph2 liver study expected early 2021) and more importantly Ph3 studies (Lung, Pancreatic and Ovarian) either stop for success or continue to completion at the interim analyses (expected in 2021); commercial performance needs to continue positive trend
- **It doesn't work if...** Optune fails to demonstrate activity outside of the approved indications (GBM, MPM) and commercial uptake shows signs of weakness; delays in clinical trial timelines could also be a potential headwind

BMRN

- **It works if...** Roctavian and vosoritide have positive clinical updates, vosoritide gets approved by the August PDUFA, Palynziq sales exceed expectations, and/or strategic interest intensifies; change to US regulatory path could be a tailwind (i.e., FDA aligns with EMA on 1-year ABR for Roctavian filing)
- **It doesn't work if...** the pipeline encounters setbacks (Ph3 Roctavian data, in particular), the Roctavian 5-year update disappoints (e.g., shows an appreciable decline in FVIII expression or an unexpected safety signal), product sales fall short of expectations, and/or updates from other achondroplasia or Hemophilia A programs are competitive

SMid Caps – 2020 Pullbacks and What Could Drive a Comeback

Sifting through some of the names that had pullbacks in 2020 and what could get them working in the new year

An incomplete sampling of stocks that pulled back in 2020 with potential to stage a comeback in 2021...

AGLE

- **It could come back if...** phase 3 PEACE data for lead asset pegzilarginase in Arginase 1 Deficiency are positive in mid-2021 (we believe the trial has a high probability of success based on the totality of prior phase 1/2 data together with a favorable biomarker-directed trial design)

AUTL

- **It could come back if...** the company outlines AUTO3 pivotal development plans, inclusive of outpatient administration (program update expected 1Q21), while maintaining compelling CR durability with longer follow-up of phase 1/2 ALEXANDER; clinical proof of concept demonstrated for AUTO4 in phase 1 interim data in TRBC1+ PTCL

DTIL

- **It could come back if...** mid-year data of additional dose cohorts in the PBCAR0191 phase 1 study (with further modified lymphodepletion regimen) show extended CART persistence and CR durability; debut phase 1 data from other pipeline programs (PBCAR20A/CLL and PBCAR269A/MM) show meaningfully durable CR rates

ITCI

- **It could come back if...** Caplyta's commercial potential in bipolar depression becomes better appreciated as we head toward a regulatory decision for that opportunity in late 2021

OYST

- **It could come back if...** lead asset OC-01 gets approved in dry eye disease (U.S. launch planned for 4Q21) coupled with better appreciation of its commercial potential in the DED marketplace (which we view as large and untapped)

RUBY

- **It could come back if...** initial data from the phase I/II study of RTX-240 provides early evidence of anti-tumor activity in addition to safety (particularly given 4-1BBL's history) and data demonstrating RTX-240's ability to stimulate the immune system

URGN

- **It could come back if...** Jelmyto sales are better than expected and arrive at a beat to consensus estimates, and patient accrual for the phase 3 ATLAS study of UGN-102 in LG-IR-NMIBC is stronger than anticipated (enrollment currently expected to complete by YE21)

2021 Buyside Survey

First, a look back at the 2020 survey predictions

Key takeaways from our 2020 outlook buyside survey (conducted in late 2019)

In last year's buyside outlook survey, 72% of respondents expected Biotech to outperform the market in 2020 (including 56% who expected it to outperform by 5-15% & 16% who expected it to outperform by $\geq 15\%$)... a prediction that ultimately came to fruition

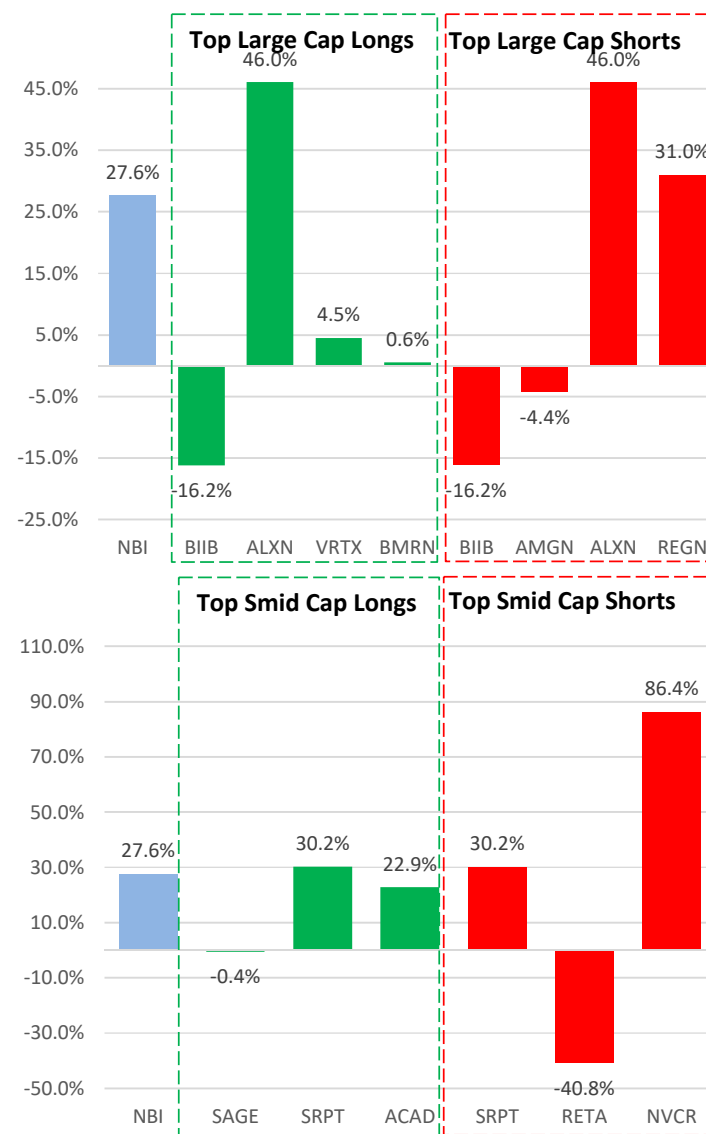
- The biotech group outperformed the broader market (YTD: NBI +28%; S&P500 +14%) after what had largely been an in-line 2019

In our 2020 outlook, the buyside voted **BIIB/ALXN (tie)**, **VRTX** & **BMRN** as top Large-Cap longs and **BIIB**, **AMGN**, **ALXN** & **REGN** as top shorts

- **Longs:** Compared with the NBI (+28% YTD), BIIB (-16% YTD), ALXN (+46% YTD), VRTX (+4% YTD) and BMRN (+1% YTD) all underperformed the index
 - Plans for AstraZeneca to acquire Alexion for \$39B (implying YTD performance of 62%) were announced over the weekend
- **Shorts:** BIIB (-16% YTD) and AMGN (-4% YTD) underperformed, while REGN (+31%) outperformed the NBI.

Respondents voted **SAGE**, **SRPT** & **ACAD** as top Smid-Cap picks in 2020 and **SRPT**, **RETA** & **NVCR** as top shorts

- Compared with its peers, only SRPT (+30% YTD) outperformed the NBI; ACAD (+23%) & SAGE (0%) underperformed the index
- Among the top shorts, RETA (-41% YTD) underperformed while SRPT (as noted above) and NVCR (+87%) outperformed



J.P.Morgan

Looking Ahead to 2021 – Buyside Survey

Key takeaways from our latest buyside survey

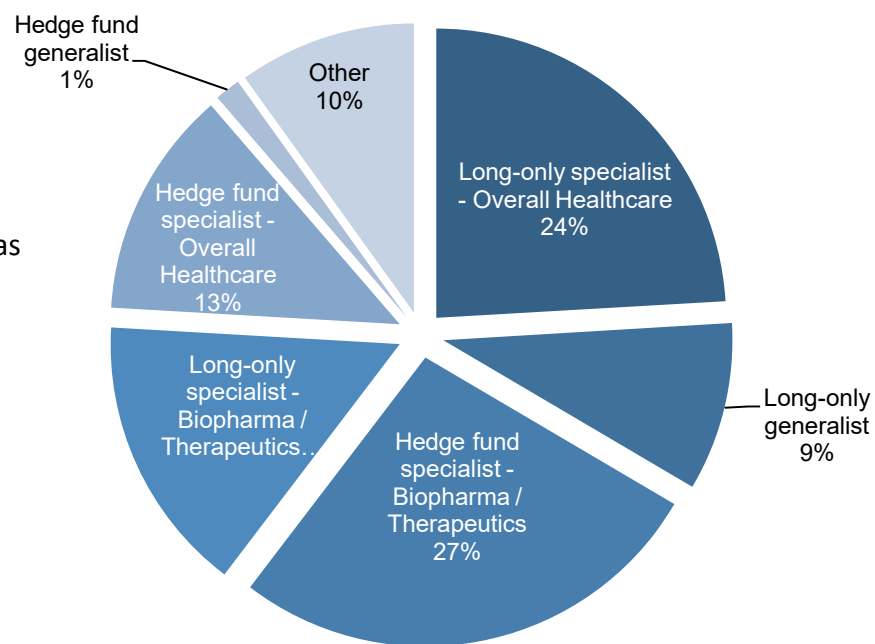
We conducted a buyside survey from Dec 3rd to Dec 10th

- 69 participants completed the survey
- Slightly more participation came from long-only funds: 49% of respondents were from long-only funds compared with 41% who were at hedge funds
- The vast majority of respondents (80%) are healthcare or biopharma “specialists”

Key takeaways include:

- 77% expect Biotech to outperform the broader markets in 2021; 12% expect the sector to underperform
- While 64% of respondents see the current regulatory environment as a tailwind, 15% see it transitioning to a headwind
- Top buyside large-cap ideas....
 - Long Idea: **BMRN**
 - Short Idea: **MRNA**
- Top buyside Smid-cap ideas....
 - Long Idea: **TGTX/GBT/SAGE**
 - Short Idea: **NVAX/ALLO/IGMS/INO**
- 79% of those surveyed expect an uptick in M&A in 2021 compared with 56% in 2020; if M&A does materialize, Acadia, Mirati and TG Therapeutics are the most commonly cited take-out candidates
- Sarepta’s ph 2 microdystrophin and Vertex’s ph 3 VX-864 (AATD) data are most frequently cited as the most important clinical catalysts in ‘21
- For the second year in a row, BIIB’s aducanumab for AD represents the most important regulatory catalyst for the coming year

Responder Type (N=69)

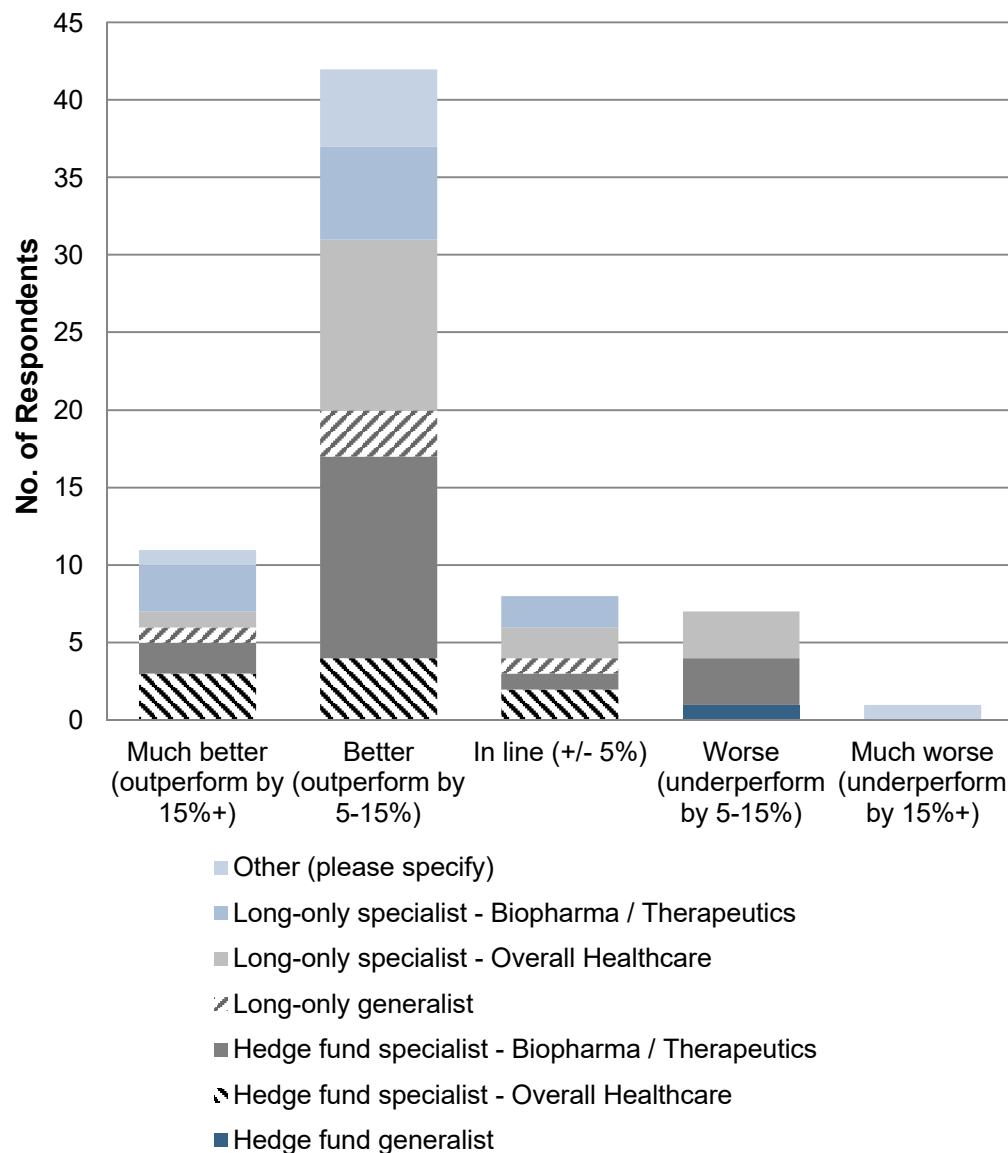
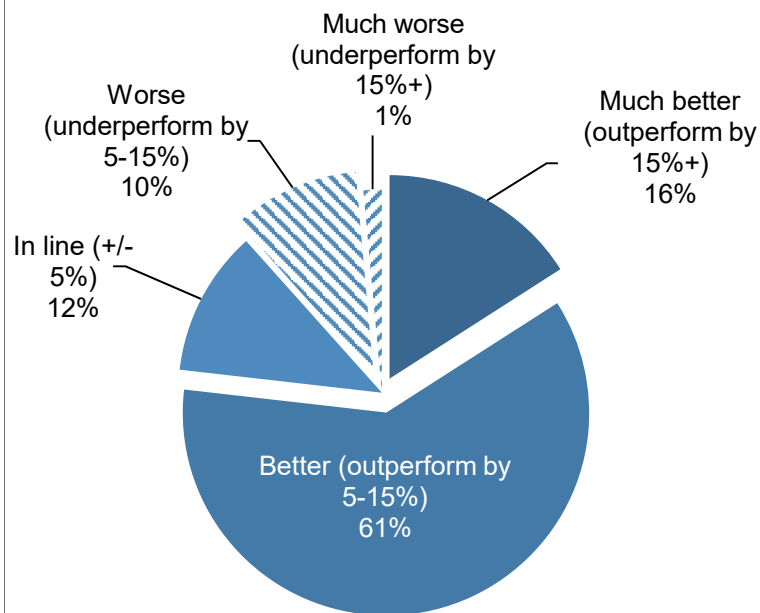


Looking Ahead to 2021 – Buyside Survey

How do you expect biotech to perform relative to the broader markets in 2021?

77% of responders expect Biotech will outperform the broader markets in 2021

- 61% of long-only investors expect Biotech will perform “better” (5-15%) and 15% expect “much better” (15%+) performance vs. 59% and 17%, respectively, for hedge funds
- 15% of long-only investors vs. 10% of hedge funds expect biotech to perform in line

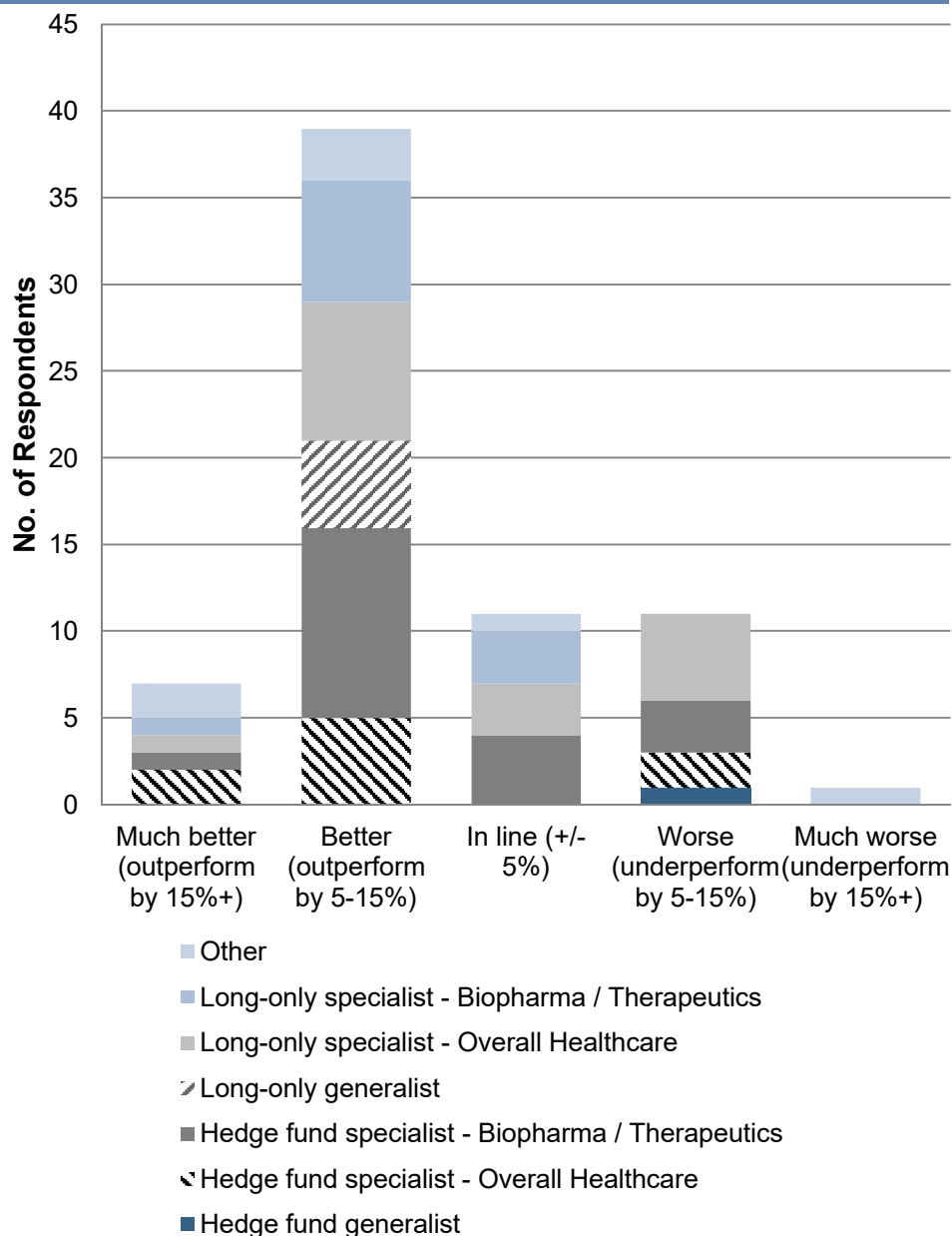
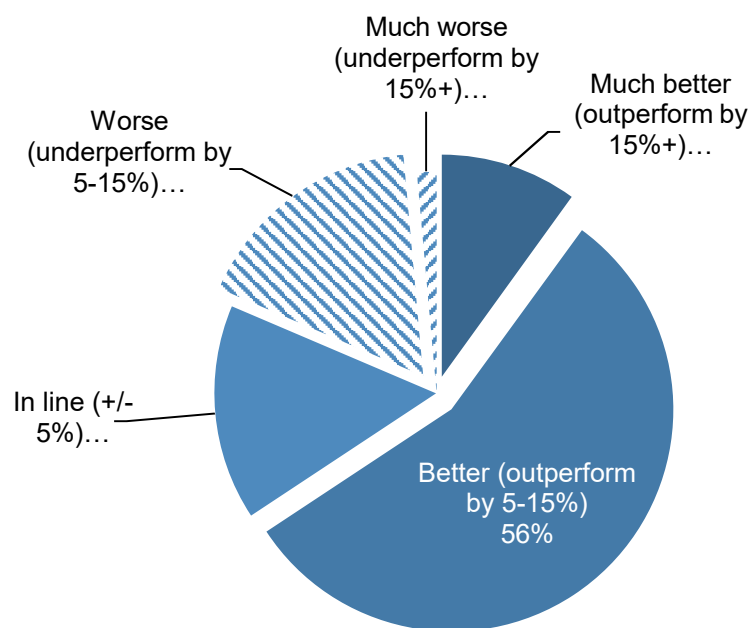


Looking Ahead to 2021 – Buyside Survey

How do you expect Biotech to perform relative to the broader Healthcare sector (Managed Care, Dist/PBM, Med Tech/Tools, Pharma) in 2021?

66% of respondents expect Biotech will outperform the broader Healthcare sector in 2021

- 61% of long-only investors expect Biotech will perform “better” (5-15%) and 6% expect “much better” (15%+) performance vs. 51% and 14%, respectively, for HFs
- 18% of long-only investors and 14% of hedge funds anticipate in-line performance with the broader healthcare sector
- 15% of long-only investors and 21% of hedge funds expect underperformance



Looking Ahead to 2021 – Buyside Survey

What is your top Large-Cap LONG headed into 2021?

Among our survey respondents, BMRN and VRTX are the top Large-Cap long picks

- In order, the top 3 picks are BMRN, VRTX, and ALXN
 - Hedge funds: 1) BMRN, 2) ALXN, and 3) VRTX
 - Long-only: 1) VRTX, 2) BMRN/SRPT/GMAB, and 3) ALXN/ALNY/HZNP
- 14% of hedge funds and 21% of long-only investors had no response

	Overall	Institution Type		Role		Expected Biotech Performance				
		Hedge Fund	Long-Only	Specialist	Generalist	Much Better	Better	In Line	Worse	Much Worse
BMRN	16%	21%	9%	16%	0%	29%	18%	0%	18%	0%
VRTX	13%	10%	18%	16%	0%	29%	13%	9%	9%	0%
ALXN	10%	17%	6%	13%	0%	0%	5%	27%	18%	0%
SRPT	7%	3%	9%	5%	17%	0%	10%	9%	0%	0%
GMAB	4%	0%	9%	5%	0%	0%	5%	9%	0%	0%
HZNP	4%	3%	6%	4%	17%	0%	5%	9%	0%	0%
ALNY	3%	0%	6%	4%	0%	14%	0%	9%	0%	0%
AMGN	3%	3%	3%	2%	17%	0%	3%	0%	9%	0%
BIIB	3%	7%	0%	4%	0%	0%	5%	0%	0%	0%
GILD	3%	3%	0%	2%	0%	14%	3%	0%	0%	0%
INCY	3%	3%	3%	4%	0%	0%	5%	0%	0%	0%
MRTX	3%	7%	0%	4%	0%	0%	3%	9%	0%	0%
ABBV	1%	3%	0%	2%	0%	0%	0%	9%	0%	0%
ARGX	1%	0%	3%	2%	0%	0%	0%	0%	9%	0%
AZN	1%	0%	0%	0%	0%	0%	3%	0%	0%	0%
JNJ	1%	3%	0%	2%	0%	0%	3%	0%	0%	0%
NVCR	1%	0%	3%	2%	0%	0%	3%	0%	0%	0%
REGN	1%	0%	0%	0%	0%	0%	0%	0%	0%	100%
ROG SW	1%	0%	3%	2%	0%	0%	0%	0%	9%	0%

Looking Ahead to 2021 – Buyside Survey

What is your top Large-Cap SHORT headed into 2021?

MRNA was the most commonly named the top Large-Cap Short, followed by BIIB

- In order, the top 3 shorts are MRNA, BIIB, and VRTX/GILD/AMGN/REGN
 - Hedge funds: 1) MRNA, 2) BIIB/REGN, and 3) VRTX/AMGN/CRSP
 - Long-only: 1) MRNA, 2) BIIB, and 3) GILD/AMGN/VRTX/AZN/ABBV
- 24% of hedge funds and 30% of long-only investors had no response

	Overall	Institution Type		Role		Expected Biotech Performance				
		Hedge Fund	Long-Only	Specialist	Generalist	Much Better	Better	In Line	Worse	Much Worse
MRNA	31%	24%	36%	32%	17%	43%	36%	27%	18%	0%
BIIB	13%	10%	15%	14%	0%	0%	10%	18%	18%	100%
VRTX	4%	7%	3%	4%	17%	0%	5%	0%	9%	0%
GILD	4%	3%	3%	2%	17%	14%	5%	0%	0%	0%
AMGN	4%	7%	3%	5%	0%	14%	3%	0%	9%	0%
REGN	4%	10%	0%	5%	0%	0%	5%	9%	0%	0%
CRSP	3%	7%	0%	4%	0%	0%	5%	0%	0%	0%
BMRN	1%	0%	3%	2%	0%	0%	3%	0%	0%	0%
JNJ	1%	3%	0%	2%	0%	0%	0%	0%	9%	0%
SRPT	1%	3%	0%	2%	0%	0%	0%	9%	0%	0%
AZN	1%	0%	3%	2%	0%	0%	3%	0%	0%	0%
ABBV	1%	0%	3%	2%	0%	0%	0%	9%	0%	0%

Looking Ahead to 2021 – Buyside Survey

What is your top Smid-Cap LONG headed into 2021?

On the Smid-cap side, TGTX/GBT/SAGE came out on top as favorite longs (though there was little consensus in Smid-cap results)

- Hedge funds: 1) TGTX/GBT/ARNA, 2) SAGE/STRO/TPTX... and many more (see table)
- Long-only: 1) SAGE/ASND, 2) TGTX/GBT/STRO/TPTX ... and many more (see table)
- 14% of hedge funds and 21% of long-only investors had no response

	Overall	Institution Type		Role		Expected Biotech Performance				
		Hedge Fund	Long-Only	Specialist	Generalist	Much Better	Better	In Line	Worse	Much Worse
TGTX	4%	7%	3%	5%	0%	0%	5%	9%	0%	0%
GBT	4%	7%	3%	5%	0%	14%	5%	0%	0%	0%
SAGE	4%	3%	6%	5%	0%	0%	8%	0%	0%	0%
STRO	3%	3%	3%	4%	0%	0%	3%	9%	0%	0%
TPTX	3%	3%	3%	4%	0%	0%	3%	0%	9%	0%
ASND	3%	0%	6%	4%	0%	0%	3%	0%	9%	0%
ARNA	3%	7%	0%	4%	0%	0%	3%	0%	9%	0%
ITCI	1%	0%	3%	2%	0%	14%	0%	0%	0%	0%
OCUL	1%	0%	3%	0%	17%	0%	3%	0%	0%	0%
PTCT	1%	0%	3%	2%	0%	0%	0%	9%	0%	0%
ACAD	1%	3%	0%	2%	0%	0%	0%	9%	0%	0%
IGMS	1%	0%	3%	2%	0%	0%	3%	0%	0%	0%
FATE	1%	0%	3%	2%	0%	0%	3%	0%	0%	0%
SRRK	1%	0%	3%	2%	0%	0%	0%	0%	9%	0%
MOR	1%	0%	3%	0%	17%	0%	3%	0%	0%	0%
CRSP	1%	3%	0%	2%	0%	0%	0%	0%	9%	0%
EXEL	1%	0%	0%	0%	0%	0%	3%	0%	0%	0%
APLS	1%	0%	0%	0%	0%	14%	0%	0%	0%	0%
NBIX	1%	3%	0%	2%	0%	0%	3%	0%	0%	0%
ZNTL	1%	3%	0%	2%	0%	0%	3%	0%	0%	0%
SBTX	1%	3%	0%	2%	0%	0%	3%	0%	0%	0%
RETA	1%	0%	0%	0%	0%	0%	0%	9%	0%	0%
RCKT	1%	3%	0%	2%	0%	14%	0%	0%	0%	0%
DVAX	1%	0%	0%	0%	0%	14%	0%	0%	0%	0%
IONS	1%	3%	0%	2%	0%	0%	3%	0%	0%	0%
CNST	1%	3%	0%	2%	0%	0%	3%	0%	0%	0%
UTHR	1%	3%	0%	2%	0%	0%	0%	9%	0%	0%
MESO	1%	0%	3%	2%	0%	0%	3%	0%	0%	0%
NBSE	1%	0%	3%	0%	17%	0%	3%	0%	0%	0%
SRPT	1%	0%	3%	2%	0%	0%	0%	0%	9%	0%
GWPH	1%	3%	0%	2%	0%	0%	0%	9%	0%	0%
ARCT	1%	0%	0%	0%	0%	0%	0%	0%	0%	100%

Looking Ahead to 2021 – Buyside Survey

What is your top Smid-Cap SHORT headed into 2021?

On the Smid-cap side, the top short pick is NVAX

- In order, the top 3 SMid shorts are 1) NVAX, 2) ALLO /IGMS/ INO, and 3) EDIT /ARCT/ MRTX
 - Hedge-funds: 1)IGMS, 2)ARCT/RARE and 3) INO/EDIT/MRTX... and a long list of names tied for third
 - Long-only: 1) NVAX, 2) ALLO/INO and 3) EDIT/ALNY/VIR... and a long list of names tied for third
- 21% of hedge fund and 52% of long-only responders did not have a response

	Overall	Institution Type		Role		Expected Biotech Performance				
		Hedge Fund	Long-Only	Specialist	Generalist	Much Better	Better	In Line	Worse	Much Worse
NVAX	6%	0%	12%	7%	0%	14%	8%	0%	0%	0%
ALLO	4%	0%	6%	4%	0%	0%	5%	0%	0%	100%
IGMS	4%	10%	0%	5%	0%	14%	3%	9%	0%	0%
INO	4%	3%	6%	5%	0%	0%	5%	9%	0%	0%
EDIT	3%	3%	3%	4%	0%	0%	3%	9%	0%	0%
ARCT	3%	7%	0%	4%	0%	14%	3%	0%	0%	0%
MRTX	3%	3%	0%	2%	0%	0%	5%	0%	0%	0%
ALNY	3%	3%	3%	4%	0%	0%	0%	0%	18%	0%
RARE	3%	7%	0%	4%	0%	0%	5%	0%	0%	0%
NVTA	1%	0%	3%	0%	17%	0%	3%	0%	0%	0%
VIR	1%	0%	3%	2%	0%	0%	3%	0%	0%	0%
INSM	1%	3%	0%	2%	0%	0%	0%	9%	0%	0%
CRSP	1%	0%	3%	2%	0%	0%	3%	0%	0%	0%
TWST	1%	3%	0%	2%	0%	0%	0%	0%	9%	0%
NTLA	1%	3%	0%	2%	0%	0%	3%	0%	0%	0%
LUN DC	1%	3%	0%	2%	0%	0%	0%	0%	9%	0%
FATE	1%	0%	0%	0%	0%	0%	3%	0%	0%	0%
NVCR	1%	0%	0%	0%	0%	14%	0%	0%	0%	0%
TRIL	1%	3%	0%	2%	0%	0%	3%	0%	0%	0%
ZIOP	1%	3%	0%	2%	0%	0%	3%	0%	0%	0%
IOVA	1%	0%	0%	0%	0%	14%	0%	0%	0%	0%
CRTX	1%	3%	0%	2%	0%	0%	3%	0%	0%	0%
RETA	1%	3%	0%	2%	0%	0%	0%	9%	0%	0%
CYDY	1%	0%	3%	0%	17%	0%	3%	0%	0%	0%
FGEN	1%	3%	0%	2%	0%	0%	0%	9%	0%	0%
RLAY	1%	3%	0%	2%	0%	0%	3%	0%	0%	0%
PTCT	1%	3%	0%	2%	0%	0%	0%	0%	9%	0%
GOSS	1%	0%	3%	2%	0%	0%	3%	0%	0%	0%
SAGE	1%	0%	3%	2%	0%	0%	0%	9%	0%	0%
NBIX	1%	3%	0%	2%	0%	0%	3%	0%	0%	0%

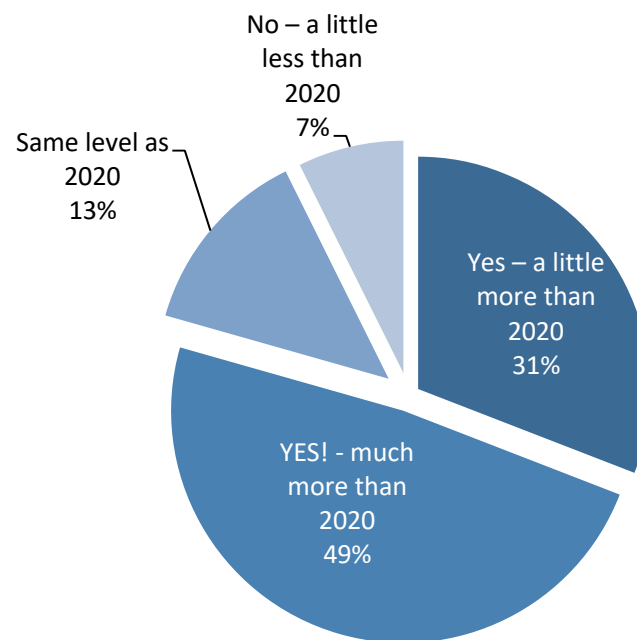
Looking Ahead to 2021 – Buyside Survey

Do you expect an increase in biotech M&A in 2021 (whole company acquisitions, not licensing deals)?

The vast majority (80%) of survey respondents expect an uptick in M&A in 2021 relative to 2020

- A similar proportion of hedge-fund and long-only responders believe there will be significantly more M&A activity in 2021 (~41-50%)

	Institution Type		Role		Expected Biotech Performance				
	Hedge Fund	Long-Only	Specialist	Generalist	Much Better	Better	In Line	Worse	Much Worse
Yes – a little more than 2020	36%	31%	32%	40%	73%	29%	0%	14%	0%
YES! - much more than 2020	41%	50%	45%	60%	27%	56%	63%	14%	100%
Same level as 2020	17%	9%	14%	0%	0%	12%	25%	29%	0%
No – a little less than 2020	7%	9%	9%	0%	0%	2%	13%	43%	0%



Looking Ahead to 2021 – Buyside Survey

If M&A were to increase, which companies might be M&A candidates?

ACAD stood out as the most frequently cited take-out candidate among survey participants for 2021, followed by MRTX & TPTX

Company	Number of Responses	Percentage (n=168*)
ACAD	9	5%
MRTX	7	4%
TPTX	6	4%
IOVA	5	3%
BPMC	5	3%
TRIL	4	2%
SRPT	4	2%
KURA	4	2%
ITCI	4	2%
INSM	4	2%
CNST	4	2%
ARNA	4	2%
ALXN	4	2%
TGTX	3	2%
STRO	3	2%
RCKT	3	2%
NBIX	3	2%
LEGN	3	2%
BMRN	3	2%
BHVN	3	2%
XLRN	2	1%
SWTX	2	1%
SAGE	2	1%
KOD	2	1%
KDMN	2	1%
HZNP	2	1%
HRTX	2	1%
FMTX	2	1%
DCPH	2	1%
CCXI	2	1%
ASND	2	1%
ALXO	2	1%
ZYME	1	1%
ZGNX	1	1%
VKTX	1	1%
VIE	1	1%
TBIO	1	1%

*Note some respondents offered more than one candidate

Looking Ahead to 2021 – Buyside Survey

If M&A were to increase, which companies might be M&A acquirers?

BIIB stood out as the most frequently cited potential acquirer among survey participants for 2021, followed closely by MRK & PFE

Company	Number of Responses	Percentage (n=175*)
BIIB	22	13%
MRK	20	11%
PFE	20	11%
AMGN	18	10%
GILD	16	9%
VRTX	13	7%
NVS	11	6%
ABBV	10	6%
SNY	8	5%
LLY	7	4%
ROG SW	6	3%
AZN	5	3%
BMJ	4	2%
GSK	4	2%
JNJ	2	1%
ALXN	2	1%
Any Large Cap BioPharma Co	2	1%
TAK	1	1%
BMS	1	1%
NVAX	1	1%
LUN DC	1	1%
REGN	1	1%

*Note some respondents offered more than one candidate

Looking Ahead to 2021 – Buyside Survey

What are the most significant CLINICAL catalysts on your radar for 2021?

SRPT's phase 2 microdystrophin gene therapy readout was cited as the most significant clinical catalyst for 2021...

- Followed by Vertex's phase 2 VX-864 data (AATD) and Sage Therapeutics's phase 3 zuranolone data for depressive disorders

Catalyst	Number of Responses	Percentage (n=89*)
SRPT microdystrophin	17	19%
VRTX AATD	12	13%
SAGE zuranolone	6	7%
APLS GA	6	7%
AlI KRAS	6	7%
JNJ vaccine	4	4%
BMRN Hema	4	4%
FOLD AT-GAA	3	3%
NVAX COVID	3	3%
ARGX CIDP	2	2%
IGMS NHL	1	1%
ROG HD	1	1%
AKRO F4 NASH	1	1%
NBIX/TKD	1	1%
ZYME BC	1	1%
TPTX ROS1	1	1%
GILD/IMMU BC	1	1%
MRK antiviral	1	1%
STRO ADC	1	1%
CARA AD-aP	1	1%
MESO COVID- ARDS	1	1%
ALXN WD	1	1%
ANAB PPP	1	1%
ALNY Cardio	1	1%
SNDX AML/ALL	1	1%
QURE HD	1	1%
BMV TYK2	1	1%
GMAB 41BB	1	1%
RTRX	1	1%
GILD Trodelvy	1	1%
BCMA GSI Data	1	1%
ASND TLR	1	1%
BBIO	1	1%
HRTX	1	1%
ATRA	1	1%
FREQ	1	1%

*Note some respondents offered more than one catalyst

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Looking Ahead to 2021 – Buyside Survey

Is there a MUST OWN name into JPM '21 in January?

- In line with their status among the top M&A take-out candidates: MRTX, TPTX, and TRIL are the must-own names heading into the 2021 J.P. Morgan Healthcare Conference

Company	Number of Responses	Percentage (n=32*)
MRTX	3	9%
TPTX	3	9%
TRIL	3	9%
TGTX	2	6%
SRPT	2	6%
IGMS	1	3%
SAGE	1	3%
IMVT	1	3%
ZYME	1	3%
VRTX	1	3%
IOVA	1	3%
MRSN	1	3%
IRWD	1	3%
GWPH	1	3%
ARCT	1	3%
PMVP	1	3%
NBI	1	3%
BMRN	1	3%
SNDX	1	3%
KOD	1	3%
KURA	1	3%
ALXN	1	3%
CCCC	1	3%
AXLO	1	3%

*Note not all respondents participated

Looking Ahead to 2021 – Buyside Survey

What are the most significant REGULATORY catalysts on your radar for 2021?

On the regulatory front, focus is not surprisingly on BIIB's aducanumab

- Following an AdCom in November, regulatory action is anticipated in 1Q21 (PFUDA date of March 7th, 2021)

The next most anticipated regulatory catalyst is ACAD's pimavanserin PDUFA (April 3rd, 2021) for DRP

Catalyst	Number of Responses	Percentage (n=68*)
BIIB aducanumab PDUFA	25	37%
ACAD pimavanserin PDDUFA	8	12%
BMRN vosoritide PDUFA	6	9%
BMRN valrox filing acceptance	4	6%
SRPT microdystrophin	4	6%
IOVA BLA submission	3	4%
BLUE bb2121 PDUFA	2	3%
RETA adcom	2	3%
BMJ/BLUE ide-cel PDUFA	2	3%
TPTX repotrectinib approval	1	1%
ARGX efgartigimod PDUFA	1	1%
AUPH PDUFA	1	1%
CCXI PDUFA	1	1%
ASND TransCon hGH PDUFA	1	1%
KPTI Selinexor PDUFA	1	1%
NVAX Vaccine	1	1%
JNJ Vaccine	1	1%
MRNA full approval	1	1%
GLPG/GILD Filgo	1	1%
BMJ TYK2	1	1%
BMJ liso-cel	1	1%

*Note not all respondents participated

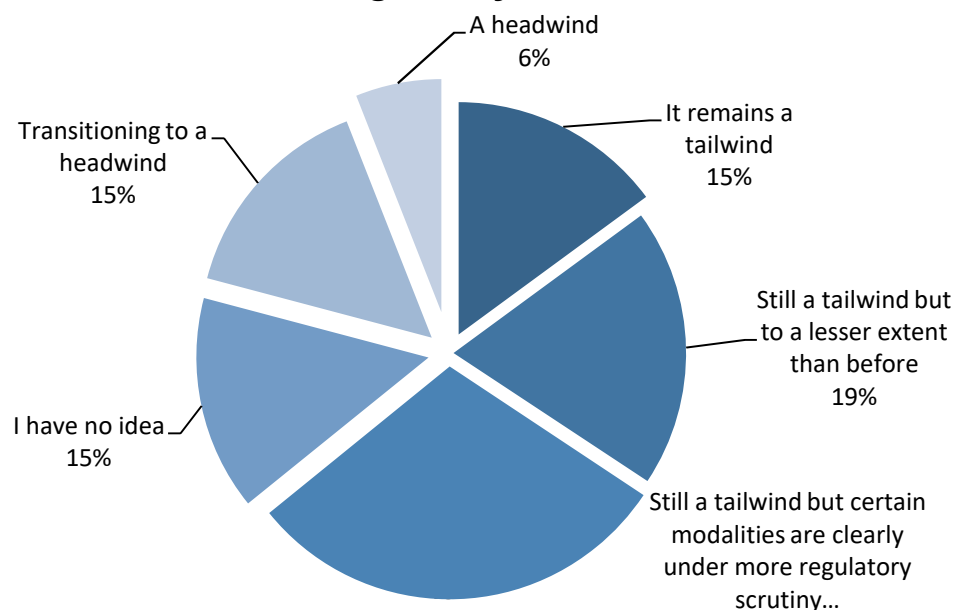
Looking Ahead to 2021 – Buyside Survey

What best describes the current US regulatory environment?

- About 2/3 of survey respondents see the current regulatory environment as a tailwind with 15% seeing it transitioning to a headwind
- A meaningful proportion of those responding 'tailwind' selected "still tailwind but certain modalities under more reg. scrutiny"

	Institution Type		Role		Expected Biotech Performance				
The Current Reg. environment...	Hedge Fund	Long-Only	Generalist	Specialist	Much Better	Better	In Line	Worse	Much Worse
Remains a tailwind	4%	28%	40%	15%	18%	15%	0%	29%	0%
Still a tailwind but to a lesser extent	21%	19%	0%	22%	27%	23%	13%	0%	0%
Still tailwind but certain modalities under more reg. scrutiny	32%	31%	20%	33%	36%	28%	63%	0%	0%
I have no idea	18%	9%	20%	13%	9%	15%	25%	14%	0%
Transitioning to a headwind	21%	6%	20%	13%	9%	13%	0%	43%	100%
A headwind	4%	6%	0%	5%	0%	8%	0%	14%	0%

The Current U.S. Regulatory Environment is...



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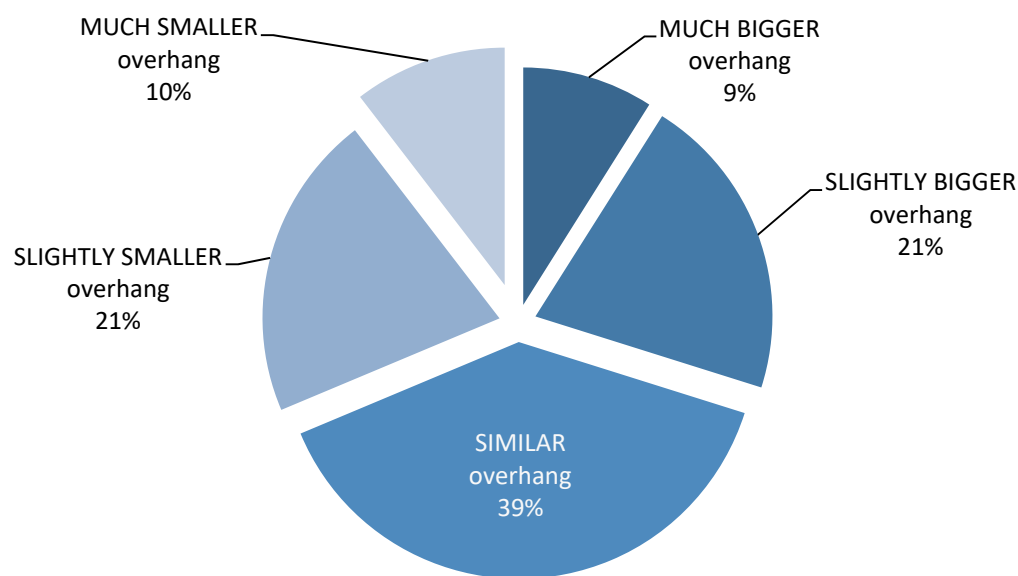
Looking Ahead to 2021 – Buyside Survey

How big of an overhang do you expect drug pricing and healthcare reform to be on biotech in 2021?

- 69% of survey respondents (in aggregate) expect pricing to be a similar or bigger overhang in 2021 relative to 2020, but the most common answer was 'similar' with ~40% and a near-equal proportion of the remainder seeing it as smaller or bigger
- Specialists are more skewed towards the middle with 44% expecting pricing to be a similar overhang

Drug pricing & HC reform in 2021 will be...	Institution Type		Role		Expected Biotech Performance				
	Hedge Fund	Long-Only	Generalist	Specialist	Much Better	Better	In Line	Worse	Much Worse
MUCH BIGGER overhang	7%	6%	20%	5%	9%	10%	0%	0%	100%
SLIGHTLY BIGGER overhang	18%	22%	20%	20%	27%	18%	25%	29%	0%
SIMILAR overhang	39%	44%	20%	44%	36%	38%	50%	43%	0%
SLIGHTLY SMALLER overhang	25%	19%	20%	22%	9%	23%	25%	29%	0%
MUCH SMALLER overhang	11%	9%	20%	9%	18%	13%	0%	0%	0%

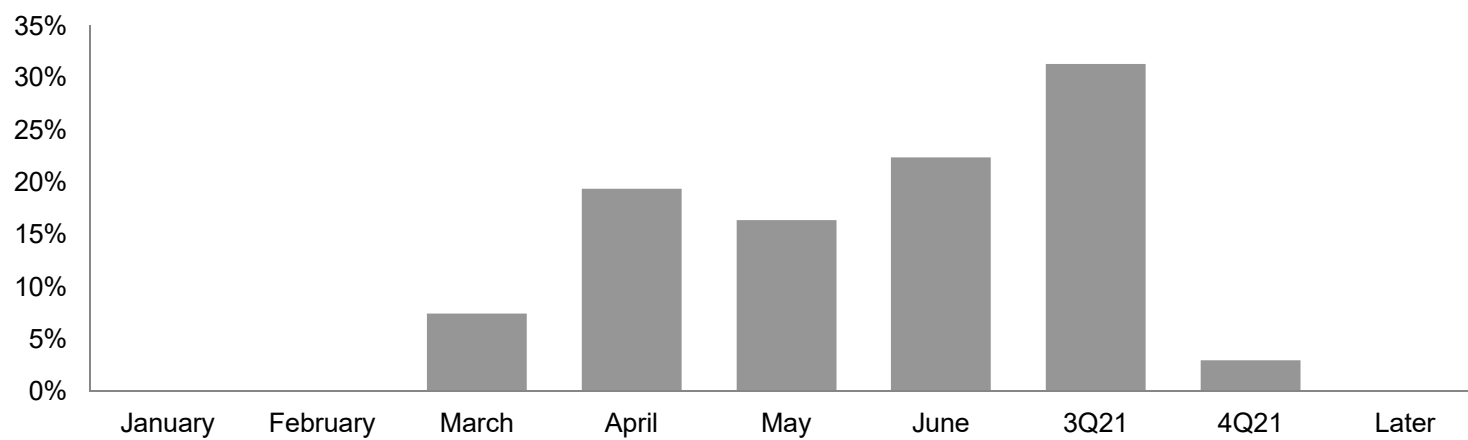
Drug pricing in 2021 will be a...



Looking Ahead to 2021 – Buyside Survey

When do you expect to personally have access to a COVID-19 vaccine?

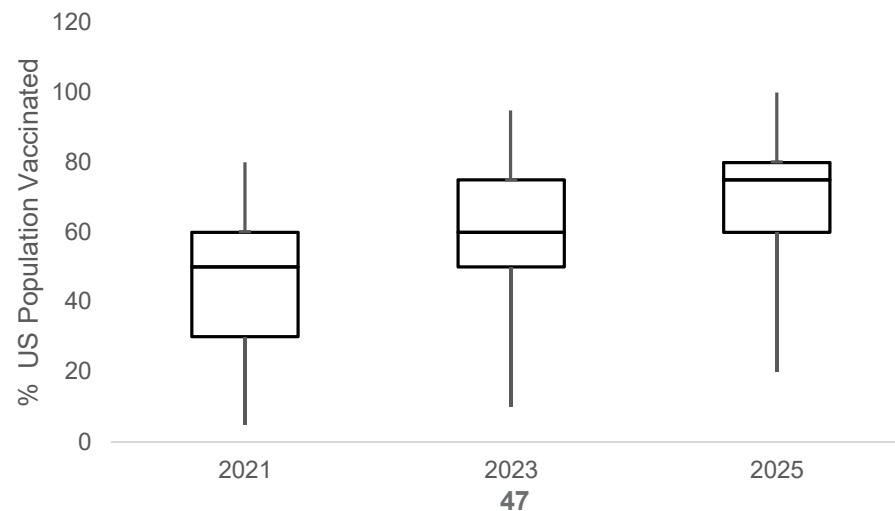
- The majority of respondents anticipate having access to a vaccine by 2Q21



What percentage of Americans do you expect to be vaccinated against COVID-19 in 2021, 2023, and 2025?

- Respondents expect vaccination rates to increase between 2021 and 2025, and >50% of Americans vaccinated by 2023

Distribution of Expectations for % Vaccinated



Looking Ahead to 2021 – Buyside Survey

Are there any overarching or specific views that you have regarding biotech in 2021 that you'd like to share?

Select Comments
generational bottom
Innovation will continue to drive value creation
Large cap biotech will continue to struggle due to lack of innovation. SMID cap biotech will do well. There will be more vaccine than subjects by mid 2021. Covid 19 will be a thing of the past by 2023
Jan 6 GA run-offs could set the tone for the year
Should be a good sector on catalyst data flow and as vaccine hysteria quiets down, sector performance should broaden
MRNA short short short
Biotech should continue to outperform the S&P and Dow due to continued attention to the sector with COVID and clinical data coming from precision medicine oncology
Is it weird that I'm going to miss JPM21 in person? And expect 1 or 2 bigger M&A deals in 2021
Go Long
Biotech is cheap within healthcare. Healthcare is cheap relative to the market and is leading the growth->value rotation. Should be a good year for biotech.
mega M&A will return

Key Themes to Monitor in 2021

- Vaccines/COVID-19
- Healthcare Reform
- Growth Expectations
- The Innovation Cycle
- Regulatory Environment
- Drug Launches
- Uses of Cash/M&A
- Capital Markets
- Concentration Risk

Executive Summary of the COVID-19 Vaccine / Therapeutic Landscape

With COVID-19 being a focal point for both biotech and the broader markets, it's useful to look at the vaccine and therapeutic landscape and the anticipated catalysts in the coming days, weeks, and months

- We view vaccines as clearly the most important modality in the fight against the COVID-19 pandemic given their protective nature and potential for widespread use (and lower cost). Early efficacy results are nothing less than impressive.
- That said, other treatments (antibodies and other anti-virals) will be needed while the pandemic persists.

COVID-19 vaccine updates will remain a key focus in the near term...

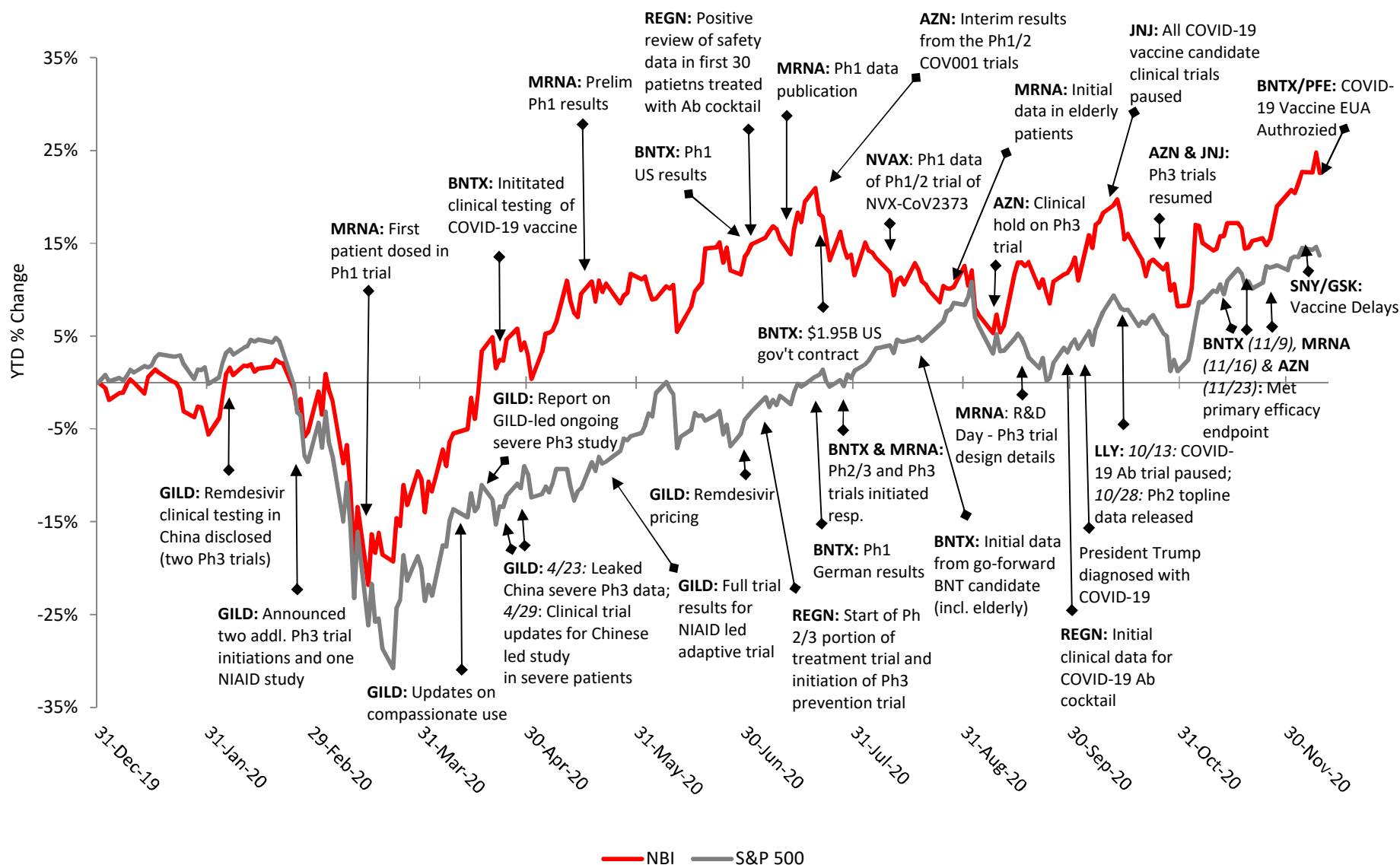
- **Final efficacy from the Phase 3 BNTX/PFE BNT162 and MRNA mRNA-1273 trials has set a high bar for competitors including JNJ/Ad26.CoVS.2 (efficacy data by end of January), AZN/AZD1222 US (data in 1Q21) and NVAX/NVX-COV2373 (UK phase 3 data in 1Q21).**
- The approvability and adoption of vaccines that fall well short of BNTX/PFE and MRNA, despite meeting FDA guidelines, is an outstanding question. Is AZD1222 at ~70% efficacy sufficient? Or will the unmet need and demand warrant approval to prevent a supply bottleneck?
- That said, continued successes on the COVID-19 vaccine development front will be a positive for the entire market, suggesting that the return to normal may be sooner than we anticipate (we believe mass vaccination in the US could occur as soon as this spring).
- If additional vaccine candidates' data are sub-par/negative, the market reaction would depend on how it reads through to other vaccines (target/virus, design, or specific platform used) and implications for supply in 2021.

The COVID-19 vaccine market is increasingly likely to be competitive as it's no longer a question of if, but rather how many vaccines will work

- **There are some 52 vaccine candidates undergoing clinical evaluation (i.e., in humans) and ~162 in preclinical development.** It's way too early to pinpoint one vaccine (or platform) as definitively best-in-class and would take a real standout on efficacy and/or safety (and maybe ultimately convenience) with so many Phase 3 readouts to come.
- BNTX/PFE and MRNA are clearly the "front runners" based on some pretty remarkable successes to date, but there are also a number of high-profile fast followers... not to mention the hundreds of additional competitors on their heels.

S&P and NBI Performance Chart with Timeline of Key COVID-19 Events

2020 YTD Performance: BNTX +229%, MRNA +653%, GILD -9%, NBI +28%, S&P 500 +14%



Summary of Key Vaccine Candidates

Vaccine	Company	Mechanism	Doses	Timing	Phase 1	Phase 1/2	Phase 2	Phase 3	Filing
BNT162	BioNTech / Pfizer	mRNA	2	0,21 days	N/A	BNT162b2 data BNT162b1 data	N/A	NCT04368728 Full Data	EUA Approved UK Authorized BLA April
mRNA-1273	Moderna	mRNA	2	0,28 days	Data Elderly Data	N/A	NCT04405076 Data expected in “coming months”	NCT04470427 Primary Topline	EUA Approval post AdCom (Dec 17)
AZD1222	AstraZeneca	AAV	2	0,28 days	N/A	Data	N/A	NCT04516746 UK Interim Data US data 1H21	
Ad26.COVS.2.S	JNJ	AAV	1 (Ph3)	Once or 0,57 days	NCT04509947	NCT04436276 Data	NCT04535453	NCT04505722 Data end Jan '21 NCT04614948	EUA early 2021
CoronaVac	SinoVac	Inactivated	2	0,14 or 28 days	N/A	Data	N/A	NCT04456595	
Ad5-nCoV	CanSinoBIO	AAV	1	Once	Data	N/A	Data	NCT04526990	
BBIBP-CorV	Sinopharm	Inactivated	2	0,21 days	N/A	Data	N/A	NCT04560881 Topline Data	
NVX-CoV2373	Novavax	Nanoparticle	2	0,21 days	N/A	Data	NCT04533399	Initiate US “coming weeks” NCT04583995 –UK UK data 1Q21	
INO-4800	Inovio	DNA	2	0,28 days	N/A	NCT04447781 NCT04336410	NCT04642638		
TBD	SNY/GSK	Protein	1 or 2	0, or 0, 21 days	N/A	NCT04537208 Topline	Initiate Feb '21	Initiate 2Q21	Earliest Approval 4Q21
CVnCoV	CureVac	mRNA	2	0,28 days	NCT04449276 Data	N/A	NCT04515147	Initiate 4Q	
V591	Merck	Viral vector	1 or 2	0, or 0, 58 or 168 days	N/A	NCT04498247			
ARCT-021	Arcturus	mRNA			N/A	NCT04480957 Interim Data			
KBP-201	Kentucky Bioprocessing	Protein	2	0,21 days	N/A	NCT04473690			
V590	Merck/IAVI	Viral vector	1	Once	NCT04569786				
SCB-2019	Clover Biopharma	Protein	2	0,21 days	NCT04405908				
MRT5500	SNY/TBIO	mRNA				Initiate 1Q21			

Key Upcoming Vaccine Timelines

		Timelines				
Company	Vaccine	Dec-20	1Q21	2Q21	3Q21	4Q21
BNTX/PFE	BNT162	EUA ✓		BLA April	Ph2/3 Adolescent Data	
MRNA	mRNA-1273	EUA				
JNJ	Ad25.COVS.S		Ph3 Data (late Jan) EUA	Two-dose Ph3 Data		
AZN	AZD1222			EUA US Phase 3 Data (1H21)		
NVAX	NVX-2373		UK Ph3 Data (1Q21) US Ph3 Initiation	Ph3 Data EUA		
SNY/GSK	TBD	Ph 1/2 Results	Ph2 Initiation (Feb)	Ph3 Initiation (2Q21)		EUA (4Q21)

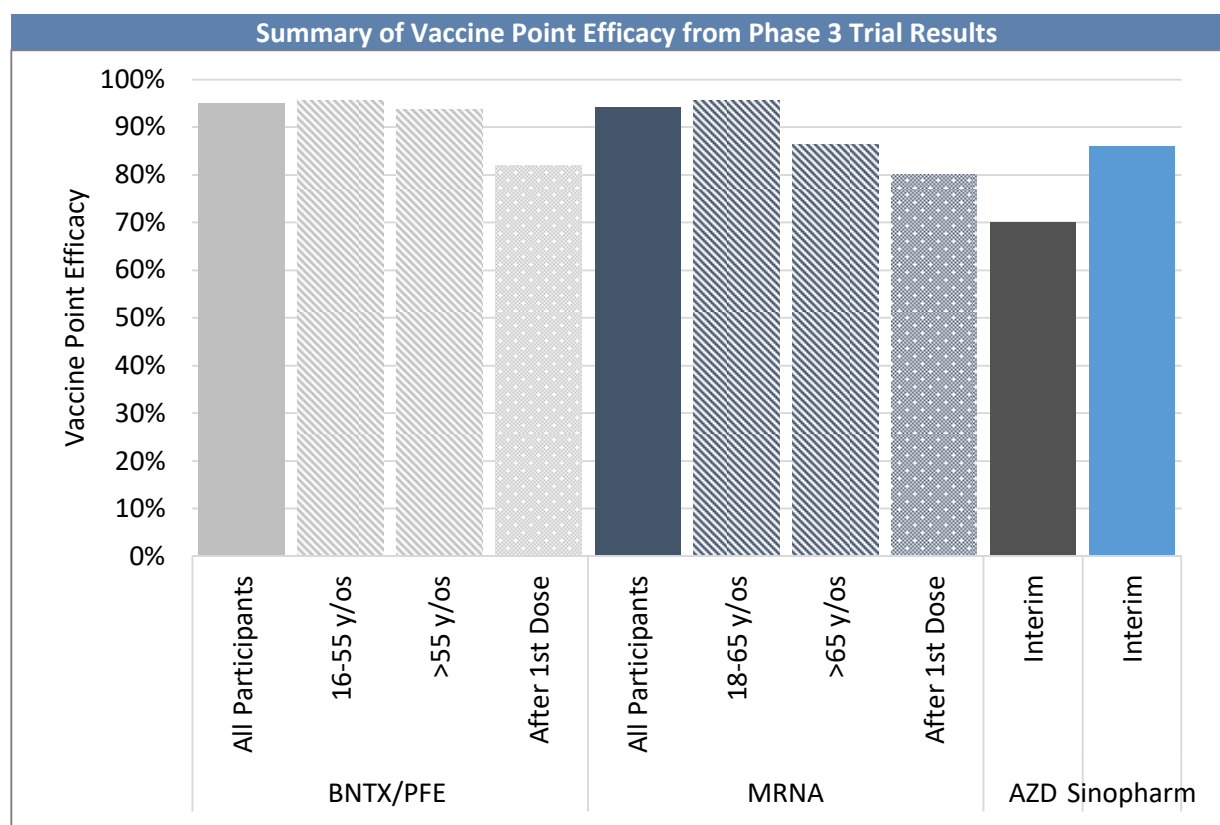
Company Guidance

JPM Estimate

Summary of Pivotal Phase 3 Vaccine Data

BNTX/PFE and MRNA have clearly set a high bar with overwhelming efficacy against COVID-19 incidence

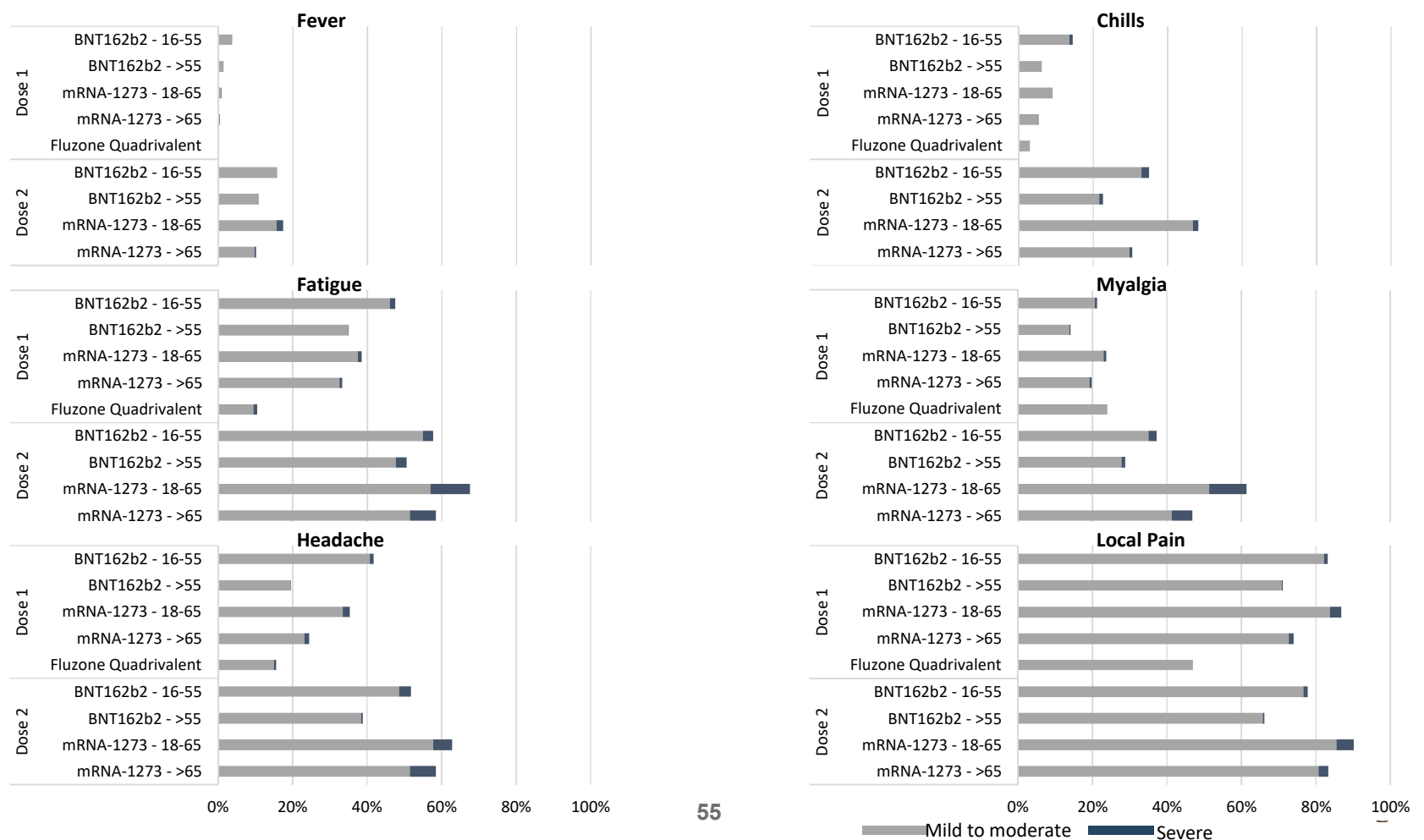
- Final analyses from the two mRNA-based vaccines are highly effective and very similar across key subgroups
- AZN/Oxford also released early results from its UK-based Phase 3 study... though they are a bit underwhelming (efficacy of 70.4%) and introduce the question of whether this is approvable in the US given the bar set by mRNA and BNTX/PFE
- Phase 3 data from Sinopharm's vaccine in a UAE-based study was also released, arriving at point efficacy of 86%
- On the heels of these first positive updates, we see a higher probability of success across the board for other vaccines, though impressive efficacy will have to be demonstrated to remain competitive



Summary of Pivotal Phase 3 Safety Data

Safety data from the briefing documents for BNTX/PFE and MRNA revealed an overall acceptable profile, though the long term consequences of an MRNA-based vaccines remain to be seen

- Between the two vaccines, the safety profile is very similar, though BNT162 may have a slight edge with the second dose
 - That said, emerging cases of anaphylaxis after BNT162 need to be monitored (no reports of anaphylaxis from mRNA-1273)
- Nonetheless, the AEs associated with these vaccines are not insignificant and could be one key area of differentiation for the number of vaccine fast-followers



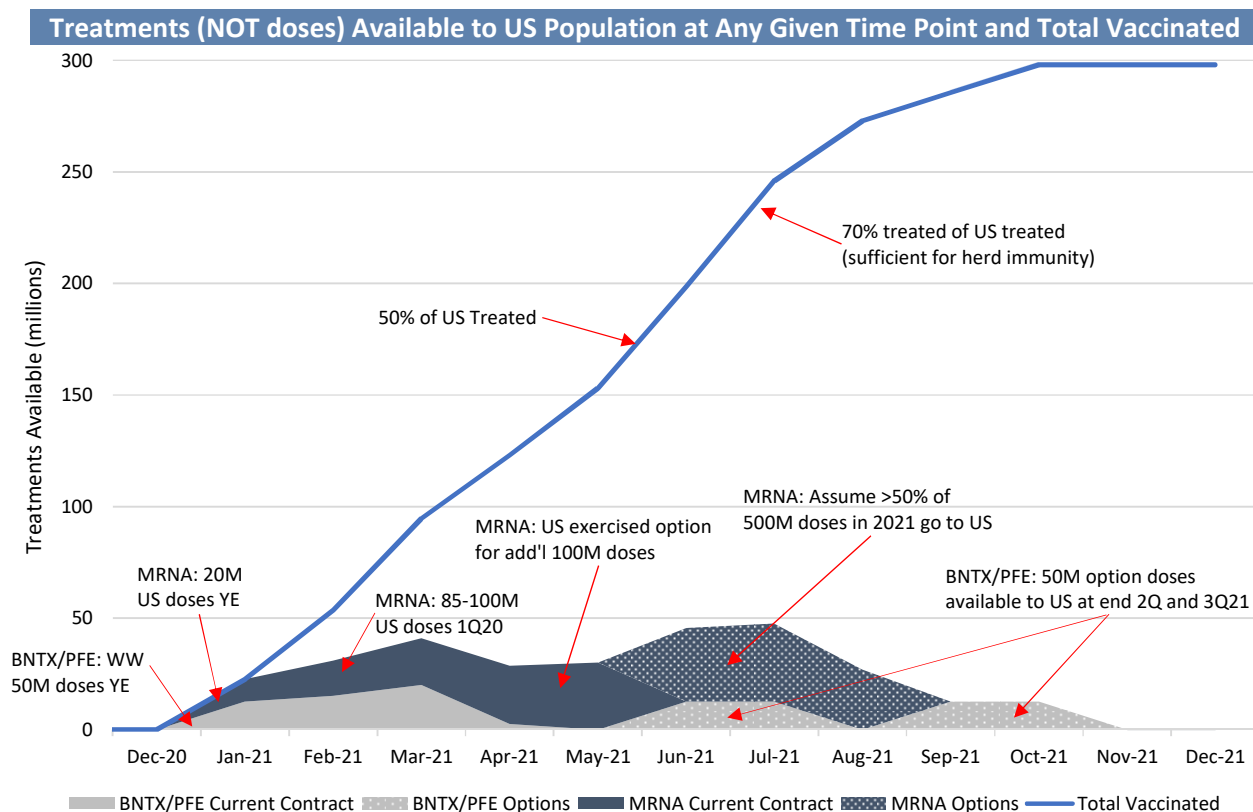
Vaccine Availability in the US

One of the more popular recent inbound questions is: “Assuming these vaccines work, when should we expect widespread vaccination of the broader population?” The short answer is, it depends... and it depends on a number of factors. Among them are:

- How many vaccines work?
- Is an EUA enough for mass distribution (vs. front-line workers / high-risk individuals) or will a full approval be required (and what will that entail)?
- How many doses of approved vaccines go to the US vs. other countries?
- How fast can production of the original, successful vaccines be scaled up?

On that last point, we created the chart below to illustrate what vaccine availability might look for BNTX/PFE and MRNA at a given time point based on company commentary and committed doses to the US. Additional companies will be added once there is more clarity on data and timelines.

- We’d note that prior [commentary](#) from Operation Warp Speed and CDC director Dr. Robert Redfield aims to have as many as 300M doses (150M treatments) available and deployed by mid-2021.



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Worldwide Manufacturing Agreements

Below are the contracts for lead vaccine candidates in various regions...

- Overall, we do not expect “vaccine nationalism,” though how vaccines are allocated amongst various countries remains to be seen
- We note that mRNA plans to supply the US with 20M doses by YE20, and 85-100M doses in 1Q21 of the 100-125M it expects to produce. The US exercised a 100M dose option to be delivered in 2Q21, with the potential to purchase an additional 300M doses.
- BNTX/PFE original contract is for 100M doses, though it is reported if the US exercises add'l options, 50M add'l BNT162 doses would not be available until end-2Q21 and then again until end-3Q21

Company – Vaccine	BNTX/PFE – BNT162		MRNA - mRNA1273		AZD - AZD1222	
Region	# of doses	Contract Value	# of doses	Contract Value	# of doses	Contract Value
US	100M; option for additional 500M	\$1.95B for first 100M doses	100M; Exercised 100 of 400M option	\$1.525B for first 100M doses	400M	\$1.2B
Canada	20M; option for additional 56M	Not disclosed	40M	Not disclosed	-	-
EU	200M; option for additional 100M	Not disclosed	80M; option for additional 80M	Not disclosed	400M	Not disclosed
UK	30M	Not disclosed	7M	Not disclosed	100M	Not disclosed
Others	Japan: 120M doses Hong Kong & Macao: 10M doses		Japan: 50M doses; Switzerland: 7.5M doses; Israel: 6M doses		CEPI/GAVI: 300M doses for \$750M LMIC: 1B doses	

Company – Vaccine	JNJ - Ad26.CoV2.S		SNY/GSK - TBD		NVAX – NVX-CoV2373	
Region	# of doses	Contract value	# of doses	Contract value	# of doses	Contract value
US	100M; option for additional 200M	>\$1B for first 100M doses	100M; option for additional 500M	\$2.1B for first 100M doses	100M	\$1.6B
Canada	38M	Not disclosed	72M	Not disclosed	76M	Not disclosed
EU	200M; option for additional 200M	Not disclosed	300M	Not disclosed	-	-
UK	30M; option for additional 22M	Not disclosed	60M	Not disclosed	60M	Not disclosed
Others	LIC: up to 500M doses with delivery beginning mid-2021		200M doses for COVAX facility		India + LMIC: >1B doses Australia: 40M doses	

What We Know About Pricing

The US gov't has contracts in place for 800M+ doses of various COVID-19 vaccine candidates at prices ranging from <\$4 to \$19.50/dose

- That is enough to supply the entire US population (~330M), assuming 2 doses each (and not even considering how many would resist vaccination)
- The bottom line on pricing is the only way we can see longer-term pricing maintained at current levels is if there are ultimately only a couple of quality options on the market, but we think that is highly unlikely with encouraging preliminary data from each of the most advanced candidates and hundreds more in development
 - Competition and capacity should drive prices even lower
 - As such, we also continue to worry that too much market value is being ascribed to this and other vaccine candidates
- An unknown specific to Moderna is what financial interest – if any – the NIH may have given the joint collaboration

Vaccine	Company	US Gov't Contract	Implied Price/Dose	Comments	Link
BNT162	BNTX / PFE	100M doses for \$1.95B	\$19.50	Option to acquire add'l 500M doses at the same price	PR
AZD1222	AZN	400M doses for \$1.2B	<\$4	BARDA contract. Includes development costs	PR
TBD	GSK/SNY	100M doses for \$2.1B	<\$10	Less than half allocated for procurement. Remainder for R&D	PR
NVX-CoV2373	NVAX	100M doses for \$1.6B	<\$8	We assume less than half is allocated for procurement (like GSK/SNY)	PR
		10M doses for \$60M	\$6	DoD Contract	PR
Ad26.COVS.S	JNJ	100M doses for >\$1B	\$10	Option to acquire 200M doses in subsequent agreement	PR
mRNA-1273	MRNA	100M doses for \$1.525B	\$15.25	Option to acquire add'l 400M doses	PR

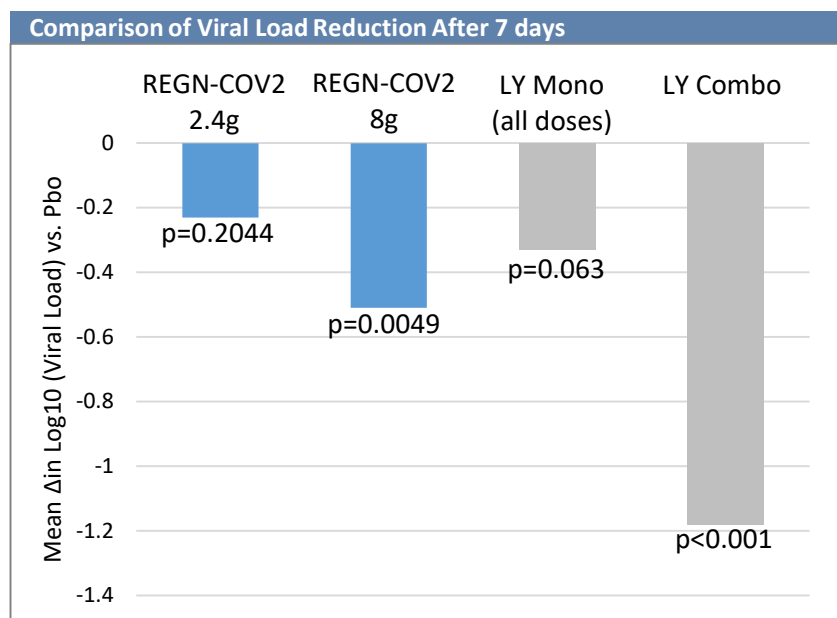
Summary of Key Neutralizing Antibody Candidates

Candidate	Company	Admin- istration	Setting	Phase 1	Phase 2	Phase 2/3	Phase 3	Filing
Covalent Sera		IV	Severe					EUA Approved
REGN-COV2	Regeneron	SQ	Prophylaxis	NCT04519437	N/A	N/A	NCT04452318	
		IV	Hospitalized	N/A	N/A	NCT04426695		
		IV	Ambulatory	N/A	N/A	NCT04425629 Data / Data		EUA Approved
LY-CoV555	LLY/AbCellera	IV	Prophylaxis	N/A	N/A	N/A	NCT04497987	
			Mild/Moderate	N/A	NCT04427501 Topline data	N/A	NCT04634409	EUA approved BLA 2Q21
			Hospitalized	NCT04411628				
LY-CoV016	LLY/Junshi	IV	Mild/Moderate	NCT04441918	NCT04427501 Data			EUA submitted
COVID-HIG	Emergent		Hospitalized	N/A	N/A	N/A	NCT04546581	
			Prophylaxis	Initiate 3Q20	Initiate 4Q20	N/A		
VIR-7832	Vir/GSK	IV	Ambulatory	N/A	N/A	NCT04545060 Data YE20		Early access 1H21
VIR-7831	Vir/GSK	IV	Ambulatory	N/A	N/A	NCT04545060	Data by YE20 Primary data Jan '21	
AZD7442	AstraZeneca	IV or IM	Prophylaxis or treatment	NCT04507256 Data in 2H20				

Summary of Key Clinical Neutralizing Antibody Data

To the extent that cross-trial comparisons can be performed, the LLY combo therapy appears to have the edge on viral load reduction while clinical outcomes (hospitalizations or ER visits) are comparable

- The LLY combination therapy (LY-CoV555 and LY-CoV016) had the greatest reduction in viral load after 7 days compared with placebo. REGN-COV2 low dose and the LLY monotherapy (LY-CoV555) did not significantly reduce viral levels compared with placebo.
- The two studies used different clinical endpoints (LLY change in symptom score and REGN time to alleviation of symptoms) making it difficult to compare across trials. However, hospitalization and ER visits were provided in each study. Overall, the rates for the placebo arms are in line (~4-6%), and there was a significant reduction for both the LLY mono and combo therapy. REGN had a numerical reduction; however, no statistics were broken out for these outcomes.



COVID-19–Related Hospitalization or ER Visits within 28 days of Treatment						
	LLY			REGN		
	Placebo (N=156)	LY Mono (All; N=309)	LY Combo (N=112)	Placebo (N=93)	REGN-COV2 2.4g (N=92)	REGN-COV2 8g (N=90)
Hospitalization or ER Visit Events (Rate)	9 (5.8%)	5 (1.6%; p=0.02)	1 (0.9%; p=0.049)	4 (4.3%)	2 (2.2%)	2 (2.2%)

Summary of Key Antivirals

Candidate	Company	Admin-istration	Mechanism	Setting	Phase 1	Phase 2	Phase 2/3	Phase 3	Filing
Remdesivir	Gilead	IV	Nucleoside analog	Hospitalized, (all)	N/A	N/A	N/A	NCT04280705 Data	Approved
				Hospitalized, severe	N/A	N/A	N/A	NCT04292899 Data	
				Hospitalized, moderate	N/A	N/A	N/A	NCT04292730 Data	
				Pediatric, severe	N/A	N/A	NCT04431453		
PTC299	PTC	Oral	DHODH inhibitor	Hospitalized	N/A	N/A	NCT04439071 Data 1H21		
Selinexor	Karyopharm	Oral	Inhibit viral nuclear export	Hospitalized	N/A	NCT04349098 Failed / Data			
AT-527	Atea	Oral	Nucleoside analog	Moderate	N/A	NCT04396106 Complete Dec			
Molnupiravir	MRK/ Ridgeback	Oral	Nucleoside analog	Mild	NCT04392219	NCT04405739 NCT04405570			
Favirpiravir	FUJIFILM	Oral	Nucleoside analog	Hospitalized	N/A	NCT04358549 (US)			
				Mild	N/A	NCT04346628 (US)		Start Sept	
PF-07304814	Pfizer	IV	Protease inhibitor	Hospitalized	NCT04535167		Start Late '20/early '21 Data 2H21		2H21
Galidesivir	Biocryst	IV	Nucleoside analog	Moderate, severe	NCT03891420				
VIR-2703	Vir/ Alnylam	?	RNAi	?	Complete preclinical YE20				

Summary of Key Anti-Inflammatory Candidates

Candidate	Company	Route	Mech	Setting	Phase 1	Phase 2	Phase 2/3	Phase 3	Filing
Dexamethasone	Generic	PO/IV	Steroid	Hospitalized	N/A		NCT04381936		Recommended
Baricitinib	LLY	Oral	JAKi	Hospitalized	N/A	N/A	N/A	NCT04421027 Topline data	EUA approved
Sarilumab	SNY/ REGN	IV	IL-6i	Hospitalized, on ventilators	N/A	N/A	N/A	NCT04315298 Failed	
				Severe/critical	N/A	N/A	NCT04327388 Failed		
Tocilizumab	Roche	IV	IL-6i	Severe	N/A	N/A	N/A	NCT04320615 Failed	
Sirukumab	JNJ	IV	IL-6i	Severe/critical	N/A	NCT04380961			
Ruxolitinib	INCY/ NVS	Oral	JAKi	Hospitalized, on ventilators	N/A	N/A	N/A	NCT04377620 Data YE20	
				COVID-19 Cytokine storm	N/A	N/A	N/A	NCT04362137 Data YE20	
TD0903	TBPH	Inhaled	JAKi	Hospitalized, ALI	NCT04350736	NCT04402866			
Ultomiris	ALXN	IV	C5 Ab	Severe pneumonia	N/A	N/A	N/A	NCT04369469	
Acalabrutinib	AZN	Oral	BTKi	Hospitalized	N/A	NCT04380688 (US)			
Ibrutinib	ABBV/ JNJ	Oral	BTKi	Hospitalized	N/A	NCT04439006			
				Hospitalized, ARDS	N/A	NCT04375397			
Mavrilimumab	KNSA	IV	GM-CSFi	Hospitalized, severe	N/A	NCT04397497	NCT04447469		
Otilimab	GSK	IV	GM-CSFi	Severe	N/A	NCT04376684 Data 1Q21			
Gimsilumab	Roivant	IV	GM-CSFi	Hospitalized, ARDS	N/A	NCT04351243			

Key Themes for 2021: Healthcare Reform (Incoming vs Outgoing)

Pricing concerns remained a hot topic on both sides of the aisle in the lead-up to (and now moving beyond) the election...

Takeaways from Biden's healthcare plan and Trump's recent executive orders

- **Both approaches attempt to address 3 areas of concern:** (1) high list prices for prescription drugs; (2) high and rising out-of-pocket costs; (3) limited access to affordable options
 - Biden's proposed policies could come into focus with congressional support on the back of the Georgia Senate runoff election outcomes favoring the democrats... while a loss to the Republicans would limit his plans

Key policies to target prescription drug pricing

- **Most favored nation/Medicare negotiations:** both have focused on the ability for Medicare to combat rising prescription drug prices
 - Trump's order would set the maximum price that Medicare Parts B and D pay for prescription drugs as the lowest price (adj. for volume and GDP difference) for a pharmaceutical product sold in other OECD countries
 - Biden's plan looks to repeal the law that prevents Medicare from negotiating with drug manufacturers, allowing it to leverage its significant purchasing weight to secure lower pricing from prescription drug makers for Part D beneficiaries as it does with hospitals
 - Similar to MFN, Biden proposes establishing an independent review board to decide the value of new drugs (with no competition) based on int'l pricing (or an independent evaluation) and set the price it will pay that private plans will also be able to access
- **Accelerate development of generics and importation of drugs:** While Biden's plan specifically mentions prohibiting extension of exclusivity beyond initial patent expirations, both aim to ease generic approvals and allow consumers to import drugs to increase competition

Biden's other proposals target drug makers' financial advantages...

- **Updated tax laws for prescription drug makers:** under this proposal, advertising for drugs would no longer be a tax deductible expense, and a tax would be imposed on manufacturers that increase the cost of their drug/biologic in excess of inflation

... while Trump looks to hit headline drugs

- **Affordable access to life-saving medicine:** the policy would set up a program for beneficiaries on public or private plans to purchase insulin and epinephrine from Federally Qualified Health Centers (FQHC) at the cost the FQHC paid
- **Eliminating kickbacks to middlemen:** the administration's policy is that discounts offered should be passed on to *patients*. The order would reset safe harbor protections to exclude retrospective reductions in price not applied at point-of-sale but add permissions for plan sponsors, pharmacies and PBMs to apply discounts at patient POS

Key Themes for 2021: Healthcare Reform

... but where do we stand now?

Takeaways from Express Scripts 2019 Drug Spending Report

- **In 2019, ESRX's Drug Trend Report (*released February 2020*) noted that patients saw an overall increase in drug spending of 2.3%, although out-of-pocket-costs were up 1.6% (vs. 0.5% increase in 2018)**
 - Spending was reduced for >33% of employers; with a 1.2% increase in spending for Medicare plans
 - The impact of brand list price inflation was muted; commercial plans saw unit costs increase 0.9% for brand drugs despite list prices of brand drugs increasing 5.2% in 2019
- **As generics enter the HIV and MS markets, unit cost declines could help offset increases expected in oncology, GI and inflammatory conditions**

Progress on pricing reform? Various proposals have been introduced, but bipartisan support remains an outstanding question

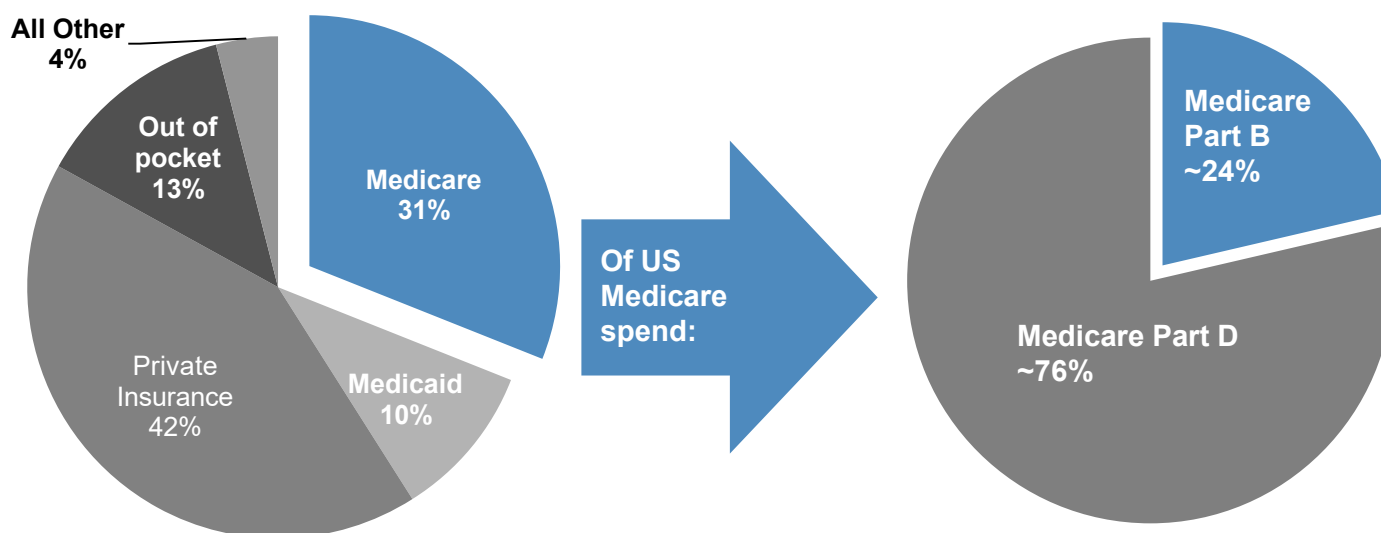
- **We highlight other proposals that are in focus now, and while we see limited potential for actual implementation in the current form, Part B IPI seems the most likely to have bipartisan support and could resurface as an overhang**
- **Elijah E. Cummings Lower Drug Costs Now Act (H.R. 3):** Proposed by Nancy Pelosi and Frank Pallone in September 2019, H.R. 3 is in line with several initiatives of Joe Biden's plan, including:
 - Allowing HHS to negotiate price on up to 250 of the most expensive branded drugs, levying penalties on non-participating drugs and/or price increases above inflation, and capping out-of-pocket costs on Medicare Part D drugs
 - The bill was passed in the House in December 2019 and has been introduced to the Senate, where it awaits scheduling for a vote
 - We see a low likelihood of H.R. 3 being passed by the Senate
- **Patient Protection and Affordable Care Enhancement Act (H.R. 1425):** Aimed primarily at improving coverage under the Affordable Care Act (ACA), the bill also includes authority for the HHS Secretary to negotiate drug prices similar to H.R. 3:
 - Estimated 10-year savings are actually ~\$70bn more than estimates for H.R. 3 due to number of drugs requiring negotiation
 - The bill was passed in the House in June 2020
 - We see a low likelihood of H.R. 1425 being passed by the Senate
- **Prescription Drug Pricing Reduction Act of 2020 (S. 4199):** In July, Senators Grassley & Wyden introduced legislation similar to their 2019 bill (S. 2543), which remained focused on:
 - Capping patient out-of-pocket costs, eliminating the coverage gap and increasing exposure in a new catastrophic phase for insurers (from 15% to 60%) and brand-name drug makers (from 0% to 20%), higher biosimilar reimbursement, and lower list price increases
 - The bill has been introduced to the Senate and awaits further action, though we see a low likelihood of success

Key Themes for 2021: Healthcare Reform

Part B exposure is relatively limited for the biopharma space as a whole

Approximately 40% of US drug revenues are paid for by the government, another ~40-45% by private insurers and ~10-15% from out-of-pocket costs

■ Note: This analysis is based on industry-wide payer mix. Individual companies may be more/less exposed to Medicare/Medicaid populations.



However, assuming a similar US reimbursement mix as the broader industry:

For Pharma:

~50% of revenues are from US Sales
~15% of revenues are exposed to US Medicare
~3.5% of revenues are exposed to Medicare Part B

For Biotech:

~70% of revenues are from US Sales
~20% of revenues are exposed to US Medicare
~5% of revenues are exposed to US Medicare Part B

On the next slide, we explore the Medicare exposure (Part B and D) across the Large-Cap universe...

Key Themes for 2021: Healthcare Reform

Relative Medicare Exposures for Large Cap Companies

2021 Medicare Part B and Part D Exposure for Large Caps*							Comments
Companies	2021 WW Sales	2021 US Sales	Part B % WW Sales Exposure	Part D % WW Sales Exposure	% NI Exposure (from Part B)	% NI Exposure (from Part D)	
AMGN	\$24,915	\$18,399	1.2%	10.5%	2.7%	23.9%	<p>AMGN provided exposure by individual product based on 2017 sales</p> <p>Spinraza has greater exposure than MS portfolio; % exposure is difficult to quantify per the company</p> <p>BMRN provided a breakdown of government/commercial exposures</p> <p>Exposure to Part B is minimal (limited to Yescarta); low double-digit exposure to Part D as per 3Q19 earnings</p> <p>Jakafi ~40% exposure to Part D; Senate draft includes a reduction in co-pays, which the company notes should include royalties and profit sharing ex-US</p> <p>30% of Adectris patients are Medicare eligible; Medicaid is in the high single digits</p> <p>CF portfolio is exposed to Part D; IR estimates about 10% exposure to Part D / 25% exposure to Medicaid</p>
BIIB	\$8,971	\$4,121	0.2%	2.5%	0.4%	6.2%	
BMRN	\$1,963	\$883	0.0%	2.3%	0.2%	10.4%	
GILD	\$22,829	\$16,939	NM	7.4%	NM	20.1%	
INCY	\$2,355	\$2,164	NM	36.8%	NM	136.0%	
REGN	\$10,332	\$8,266	2.9%	NM	5.8%	NM	
SGEN	\$1,527	\$1,309	2.6%	NM	NM	NM	
VRTX	\$6,589	\$4,612	NM	7.0%	NM	12.1%	

2025 Medicare Part B and Part D Exposure*						
Companies	2025 WW Sales	2025 US Sales	Part B % WW Sales Exposure	Part D % WW Sales Exposure	% NI Exposure (from Part B)	% NI Exposure (from Part D)
AMGN	\$27,415	\$20,724	5.2%	10.9%	10.6%	22.0%
BIIB	\$14,662	\$9,384	0.6%	0.7%	1.3%	1.6%
BMRN	\$4,735	\$2,131	0.2%	0.0%	0.4%	0.0%
GILD	\$23,349	\$17,325	NM	7.4%	NM	19.3%
INCY	\$4,162	\$3,496	NM	28.0%	NM	51.0%
REGN	\$10,872	\$7,828	11.7%	0.0%	24.2%	0.0%
SGEN	\$3,925	\$3,925	9.8%	NM	22.3%	NM
VRTX	\$8,474	\$5,250	NM	6.2%	NM	10.4%

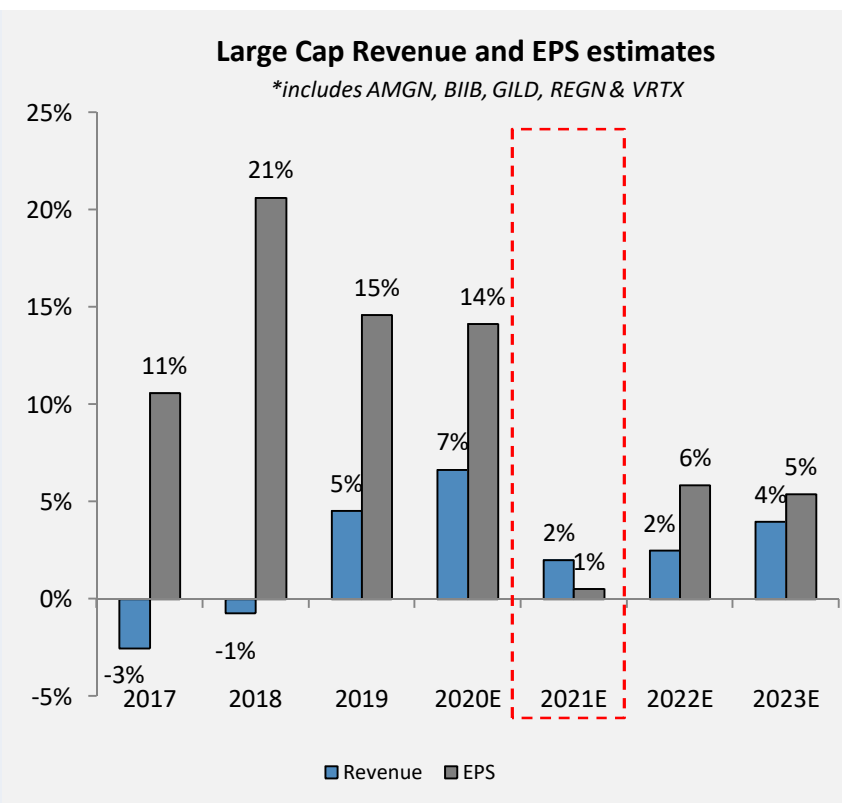
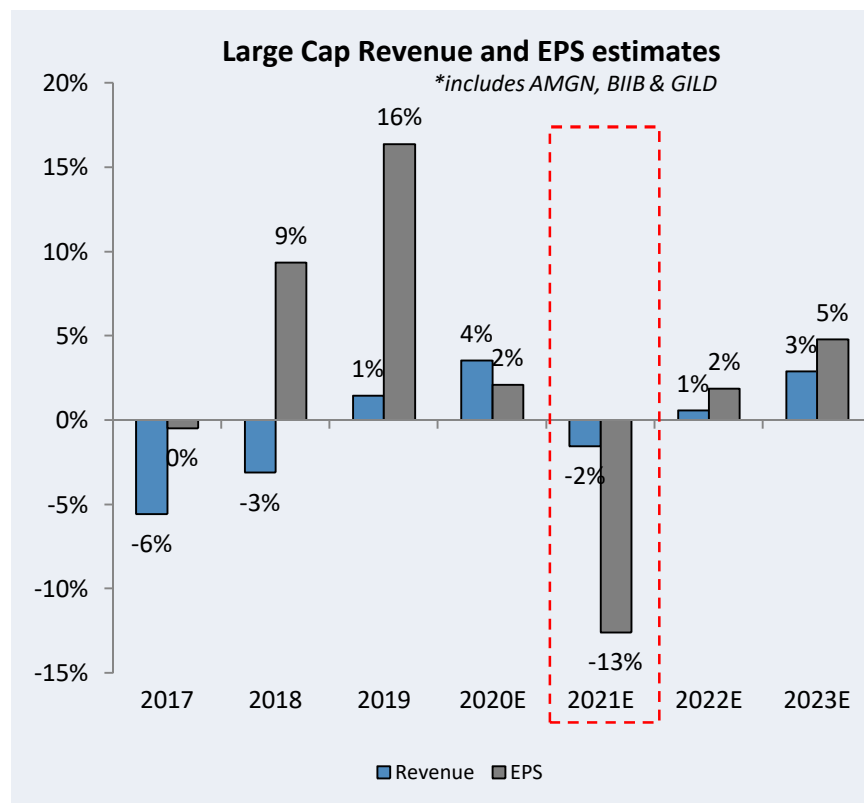
*Note that 2021 Part B exposure assumes 50% of the eligible patients and 20% adjustment for first year of implementation; 2025 Part B reflects full implementation

Key Themes for 2021: Growth Expectations

Growth expectations for 2021 remain low for large-caps

Consensus 2021 forecasts for AMGN, BIIB & GILD combined for a 2% revenue decline. Even if we include REGN & VRTX, a mere 2% growth is expected in 2021, but picking up slightly in the out years

- Bloomberg consensus calls for 13% EPS decline for AMGN, BIIB & GILD combined, which highlights one of the key issues/concerns with the sector and the driving force behind much needed M&A
- As 2021 estimates look discouraging, growth drivers are certainly needed in the medium-to-long term for these three large caps
- Not surprisingly, more growth is expected to come from the other large-caps as new launches continue to ramp-up significantly

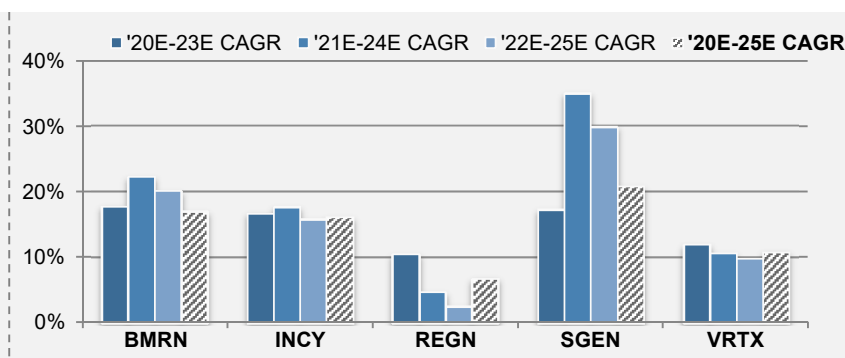
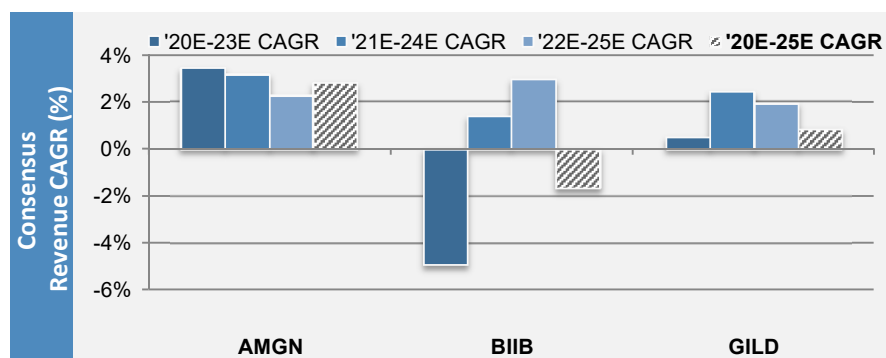


Key Themes for 2021: Growth Expectations

Looking forward, biotech offers a mixed bag of growth prospects

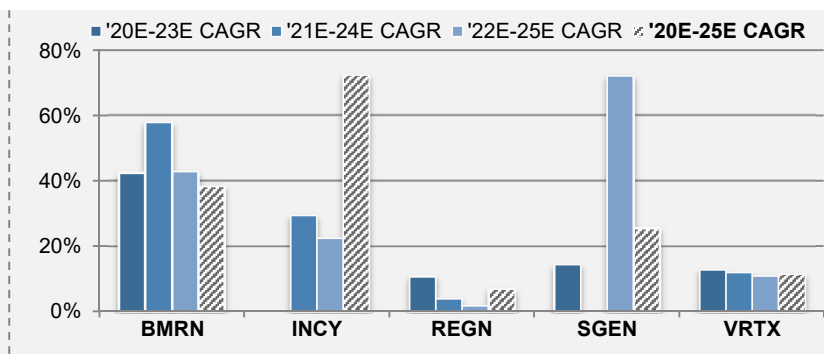
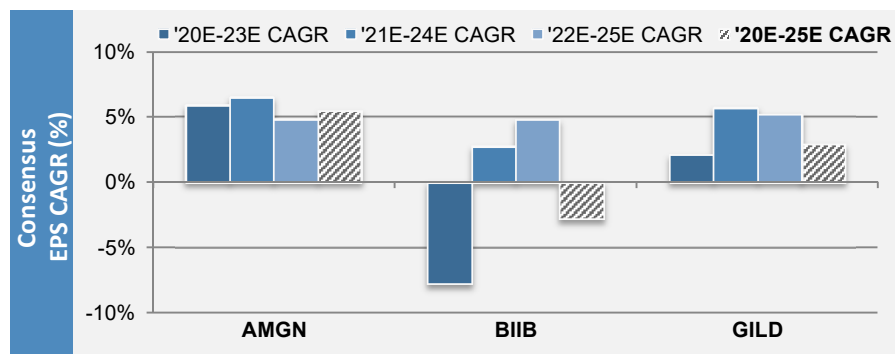
As expected, AMGN, BIIB & GILD lack revenue growth drivers, whereas others in general have double-digit CAGRs (3-yr & 5-yr)

- Emerging large caps with lower revenue bases coupled with the expected ramp-up of new products is driving higher expectations
- BMRN, INCY, SGEN, and VRTX all have attractive top-line growth prospects over the next 5 years



Higher expected top-line growth translates to higher bottom-line prospects as evident in EPS CAGRs

- BMRN, INCY, SGEN, and VRTX continue to be the major growth engines for large caps
- AMGN makes the cut among legacy large caps with a 5% EPS CAGR projected over the next five years



Key Themes for 2021: Growth Expectations

		Revenue						YoY Growth
		2020E	2021E	2022E	2023E	2024E	2025E	Trajectory
Large Caps – Revenue Consensus #s	AMGN	\$25,328	\$26,354	\$27,212	\$28,042	\$28,941	\$29,106	
	BIIB	\$13,381	\$11,395	\$11,266	\$11,509	\$11,873	\$12,293	
	BMRN	\$1,851	\$1,930	\$2,335	\$3,021	\$3,529	\$4,050	
	GILD	\$23,622	\$23,622	\$23,282	\$24,019	\$25,433	\$24,699	
	INCY	\$2,510	\$2,843	\$3,410	\$3,992	\$4,635	\$5,300	
	REGN	\$8,541	\$10,240	\$11,011	\$11,527	\$11,758	\$11,883	
	SGEN	\$2,155	\$1,807	\$2,537	\$3,479	\$4,434	\$5,547	
	VRTX	\$6,149	\$6,961	\$7,764	\$8,635	\$9,420	\$10,289	
		EPS						YoY Growth
		2020E	2021E	2022E	2023E	2024E	2025E	Trajectory
Large Caps – EPS Consensus #s	AMGN	\$16.2	\$17.0	\$18.3	\$19.2	\$20.6	\$21.1	
	BIIB	\$33.4	\$25.5	\$25.2	\$26.2	\$27.6	\$28.9	
	BMRN	\$1.6	\$1.6	\$2.8	\$4.7	\$6.5	\$8.2	
	GILD	\$6.6	\$6.6	\$6.6	\$7.0	\$7.8	\$7.6	
	INCY	-\$0.7	\$3.6	\$5.1	\$6.4	\$7.9	\$9.3	
	REGN	\$30.5	\$36.9	\$40.2	\$41.5	\$41.6	\$42.4	
	SGEN	\$3.3	\$0.0	\$2.0	\$4.9	\$7.4	\$10.1	
	VRTX	\$10.4	\$11.5	\$13.0	\$14.8	\$16.1	\$17.8	

Key Themes for 2021: Innovation

Innovation is the backbone of biotech and should continue to drive the sector in 2021 and beyond

The past 5+ years have seen significant innovation in biotech, particularly in “hot” areas such as oncology and orphan disease, and we think new and innovative products/technologies will continue to be crucial to drive innovation in the sector in 2021

Key areas to watch include (but are not limited to) gene therapy and editing / CAR-T / immuno-oncology / targeted oncology, CNS, orphan diseases, and cardio-metabolism

- **Gene Therapy / Editing / Cell Therapy:** Given the potential curative promise that comes with these approaches, we expect updates to continue in 2021 – additional de-risking data this year have helped drive investor enthusiasm for these emerging technologies. Companies to watch in the JPM universe: ALVR, ATRA, AUTL, BEAM, BLUE, DTIL, EDIT, FOLD, FRLN, LEGN, PASG, PGEN, NRIX, ORTX, RCKT, RUBY, SRPT, SLDB, SGMO, etc.
- **CNS:** There continues to be significant investment in CNS-related disorders with huge unmet medical need (e.g., Alzheimer’s, Parkinson’s, HD, ALS, MS, etc.)
 - Companies to watch in the JPM universe: ACAD, ANNX, ATRA, APTX, BIIB, DNLI, ITCI, LXR, NBIX, PASG, IONS, PTCT, SAGE
- **Oncology:** Companies are pouring into the targeted molecule and I/O oncology spaces, and sequencing of the human genome has led to significant advances in cancer treatment as “personalized” therapies become a reality
 - Targeted therapy oncology companies to watch in the JPM universe: AMGN, AGIO, APRE, DCPH, FPRX, GTHX, IDYA, IMGN, KPTI, MRSN, MRTX, ORIC, RLAY, RVMD, SGEN, SWTX, TGTX, YMAB, ZYME, ZLAB
 - I/O companies to watch in the JPM universe: IDRA, ITOS, NKTR, NRIX, REPL, REGN, RUBY (and many, many more)
- **Orphan diseases:** There continues to be innovation in the orphan world in established disease areas (e.g., Fabry, Pompe, PNH) and especially in newer indications (e.g., achondroplasia, DMD, Batten Disease, SMA, Friedreich’s Ataxia, MPS II, etc.)
 - Companies to watch in the JPM universe: AGLE, ALNY, ASND, APLS, BCRX, BMRN, CCXI, CRNX, IONS, DYNE, FOLD, FRLN, MGTA, ORTX, UTHR, STOK, SRPT, PTCT, RCKT, RARE, SGMO, etc.

Key Themes for 2021: Regulatory Environment

Average approvals in recent years are higher than in the past, underscoring favorable regulatory environment (a trend we expect to continue)

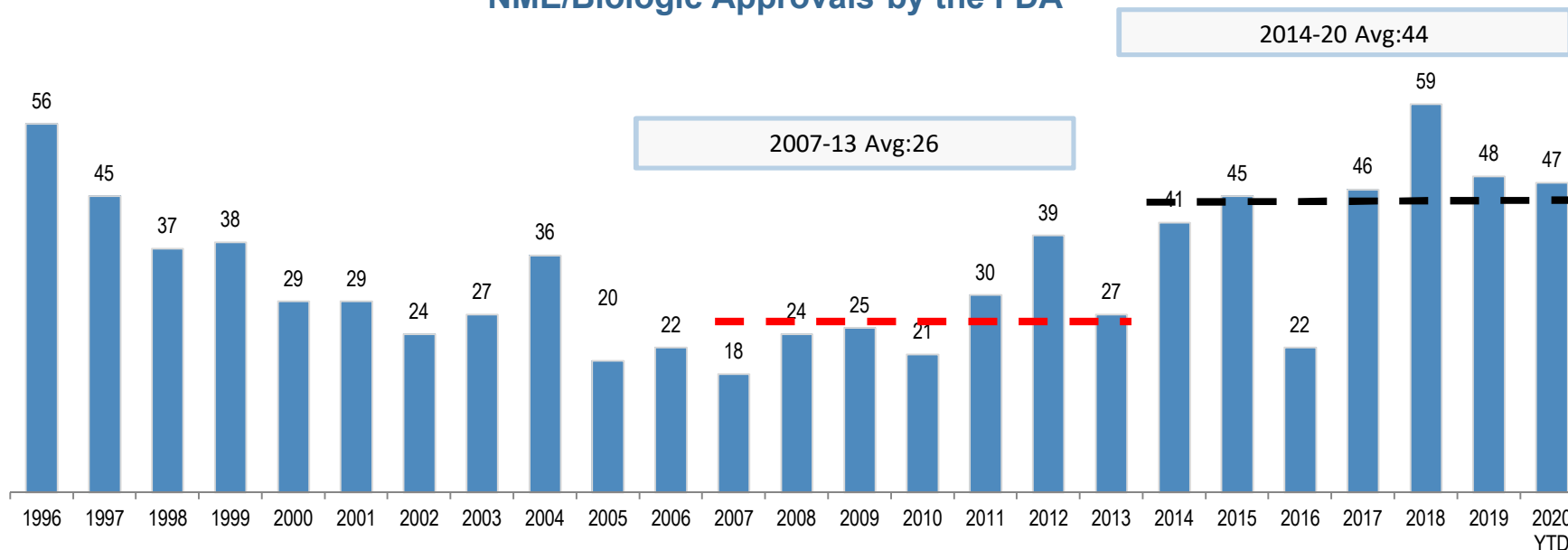
The rate of FDA approvals is similar in 2020 as compared with 2019 (though there are a few weeks left), which marks the fourth consecutive year with 45+ NME/biologic approvals.

- Average approvals since 2014 are higher than in prior years, underscoring the favorable regulatory environment.
- We expect the trend to continue and are encouraged by the FDA's increased clarity on regulatory requirements / expedited approaches.
 - That said, there seems to be more scrutiny on cell/gene therapies than in prior years... a trend that we'll closely watch.

The FDA (and Congress) remains committed to expedited drug development where appropriate.

- The FDA granted 151 Fast Track designation requests in CY 2019 (the last cut of data available), up from 145 in 2018 (and 115 in 2019).
- There were 5 Accelerated Approvals granted under Subpart H in 2020 (as of 6/20/20) (vs. 5 in 2019, 8 in 2018 and 7 in 2017).
- The FDA highlights the continued success of its expedited approval pathways, exceeding the goal of a 95% approval rate for 510(k) submissions by issuing a decision on 99% of submissions received in the FY 2019 cohort within 90 FDA days under MDUFA.
- **We expect the FDA's programs will continue to meaningfully accelerate development/approval for key products in 2021.**

NME/Biologic Approvals by the FDA



Key Themes for 2021: Drug Launches

Drug launches remain a key theme for the sector and provide further validation of innovation

Transformative products meeting unmet needs in large markets not surprisingly performed well out of the gate

- Overall, commercial execution, combined with better management of expectations by companies, has helped the perception around drug launches
- That said, dependent on the market/product, “short the launch sentiment” can still persist

COVID-19 has added another wrinkle to this dynamic due to its negative impact on patient accessibility to medication, physician-patient interactions as well as traditional relationships between physicians and company representatives

- Although we do not believe this will discourage broader investment in the sector as COVID-19 is (likely) a transient event, it could impact launch expectations in the near term
- Generally speaking, we would say that commercial biotechs have effectively managed launches/expectations through the pandemic

We expect drug launches to continue to be a key point of focus in 2021

- Key watch lists on the following slides...

Key Themes for 2021: Drug Launches (Continued)

Investor focus will remain on a number of higher-profile drug launches

	Drug	Company	Launch Date	2021E		6mo	cons trend	2022E		6mo	cons trend
				JPMe	Cons	cons % Δ		JPMe	Cons	cons % Δ	
Kasimov	vosoritide	BMRN	2H21*	\$23M	\$25M	-38%		\$154M	\$134M	-17%	
	Sotorasib	AMGN	2H21*	\$0M	\$18M	71%		\$68M	\$149M	83%	
	TV	SGEN	2H21*	\$20M	\$19M	-64%		\$160M	\$98M	-10%	
	Cilta-cel	JNJ / LEGN	2H21*	\$37M	NA	NA		\$380M	NA	NA	
	Ide-cel	BMJ / BLUE	1Q21*	\$46M	\$98M	65%		\$79M	\$213M	53%	
	BNT162	PFE / BNTX	4Q20*	\$3409M	\$5233M	1288%		\$6278M	\$6848M	12557%	
	mRNA-1273	MRNA	4Q20*	\$3341M	\$7320M	NA		\$4174M	\$6891M	NA	
	REGN-CoV2	REGN	4Q20*	\$1086M	NA	NA		\$2672M	NA	NA	
	Veklury / remdesivir	GILD	2Q20	\$2191M	\$1858M	-18%		\$604M	\$1249M	-56%	
	Tukysa	SGEN	2Q20	\$278M	\$311M	65%		\$451M	\$516M	32%	
	Padcev	SGEN	4Q19	\$338M	\$430M	7%		\$680M	\$632M	-1%	
	Trikafta	VRTX	4Q19	\$4808M	\$4833M	8%		\$5770M	\$5833M	8%	
	Oxbryta	GBT	4Q19	\$218M	\$258M	8%		\$433M	\$458M	-12%	
	Libtayo	REGN / SNY	4Q18	\$420M	\$501M	-60%		\$711M	\$762M	-55%	
	Palynziq	BMRN	3Q18	\$302M	\$295M	-2%		\$426M	\$412M	-2%	
	Crysvita	RARE	2Q18	\$265M	\$186M	5%		\$315M	\$270M	12%	
	Aimovig	AMGN	2Q18	\$537M	\$556M	-3%		\$774M	\$664M	-15%	
	Symdeko	VRTX	1Q18	\$535M	\$627M	-8%		\$491M	\$609M	-8%	
	Biktarvy	GILD	1Q18	\$8008M	\$8153M	-1%		\$8595M	\$8837M	-2%	
	Yescarta**	GILD	4Q17	\$713M	\$740M	188%		\$808M	\$968M	174%	
	Nerlynx	PBYI	3Q17	\$239M	\$230M	-10%		\$262M	\$240M	-9%	
	Dupixent	REGN / SNY	1Q17	\$5090M	\$5507M	13%		\$5938M	\$6502M	8%	

*expected launch date

** Note that Yescarta's consensus is median based, not mean.

Key Themes for 2021: Drug Launches (Continued)

	Drug	Company	Launch Date	2021E		6mo	cons trend	2022E		6mo	cons trend
				JPM	Cons	cons % Δ		JPM	Cons	cons % Δ	
Fye	Remodulin	UTHR	2021 (Pump)	\$449M	\$473M	12%		\$383M	\$436M	17%	
	Erwinaze/JZP-458 [#]	JAZZ	2021	\$42M	\$70M	-34%		\$112M	\$87M	13%	
	TransCon hGH ^{###}	ASND	2021	\$19M	\$55M	-21%		\$94M	\$186M	-13%	
	Orladeyo ^{###}	BCRX	4Q20	\$28M	\$62M	13%		\$103M	\$123M	13%	
	Xywav	JAZZ	4Q20	\$266M	\$256M	8%		\$867M	\$702M	34%	
	Zepzelca	JAZZ	3Q20	\$77M	\$147M	171%		\$175M	\$208M	82%	
	Darzalex	HALO/JNJ	2Q20 (SC)	\$5,331M	\$5,058M	3%		\$6,397M	\$7,206M	1%	
	Qinlock ^{###}	DCPH	2Q20	\$98M	\$106M	52%		\$244M	\$231M	17%	
	Nexletol/Nexlizet ^{###}	ESPR	1Q20	\$85M	\$102M	-55%		\$186M	\$331M	-20%	
	Caplyta ^{###}	ITCI	1Q20	\$112M	\$117M	-16%		\$250M	\$293M	-37%	
	Vascepa ^{###}	AMRN	4Q19	\$684M	\$708M	-20%		\$438M	\$710M	-6%	
Rama	Sunosi	JAZZ	3Q19	\$62M	\$72M	-2%		\$118M	\$130M	-2%	
	Rilonacept ^{###}	KNSA	2021*	\$4M	\$16M	-20%		\$84M	\$91M	21%	
	Trilaciclib	GTHX	3Q21*	\$11M	\$17M	-34%		\$40M	\$40M	-51%	
	Avacopan	CCXI	3Q21*	\$14M	\$44M	7%		\$142M	\$127M	4%	
	Libmeldy	ORTX	1H21*	\$6M	\$9M	102%		\$33M	\$27M	25%	
	Oxlumo	ALNY	4Q20	\$27M	\$43M	8%		\$66M	\$101M	16%	
	Ongentys	NBIX	3Q20	\$26M	\$39M	-16%		\$38M	\$81M	-13%	
	Optune	ZLAB	Mid 2020	\$48M	\$69M	-13%		\$101M	\$155M	-6%	
	Zejula	ZLAB	Early 2020	\$63M	\$73M	41%		\$112M	\$120M	8%	
	Givlaari	ALNY	4Q19	\$123M	\$141M	-5%		\$173M	\$261M	-13%	
	Onpattro	ALNY	3Q18	\$431M	\$454M	-10%		\$511M	\$594M	-15%	
Joseph	Tibsovo	AGIO	3Q18	\$142M	\$155M	-10%		\$154M	\$197M	-18%	
	Umbralisib ^{###}	TGTX	1H21	\$42M	\$54M	-53%		\$221M	\$237M	17%	
	Relugolix ^{**}	MYOV	1Q21	\$118M	\$123M	NA	NA	\$370M	\$379M	NA	NA
	Jelmyto ^{###}	URGN	2Q20	\$46M	\$54M	-12%		\$84M	\$118M	-18%	
	Evrysdi	PTCT	3Q20	\$592M	\$346M	139%		\$1,172M	\$708M	71%	
	Reblozyl	XLRN	4Q19	\$601M	\$417M	44%		\$1,064M	\$634M	0%	
	Xpovio	KPTI	3Q19	\$180M	\$155M	-23%		\$394M	\$298M	-18%	
	HCV (Mavyret / Viekira)	ENTA/ABBV	3Q17	\$1,493M	\$1,022M	-25%		\$1,281M	\$928M	-25%	

Note: Darzalex estimates represent total sales to JNJ (covered by JPM analyst Chris Schott). HALO receives a MSD royalty on the SC portion of these sales

Note: UTHR Remodulin sales are for the overall franchise while launch date cited is for RemU nity (DEKA) pumps

*JPM estimate

**Reflects estimates for FY2021 and FY2022 given company YE - March

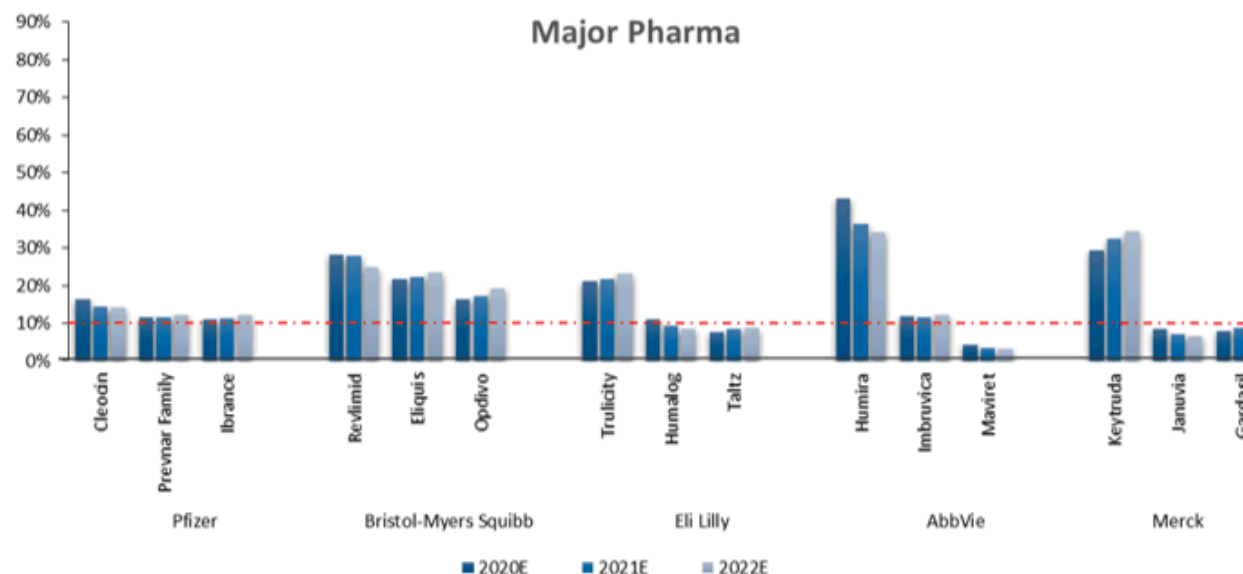
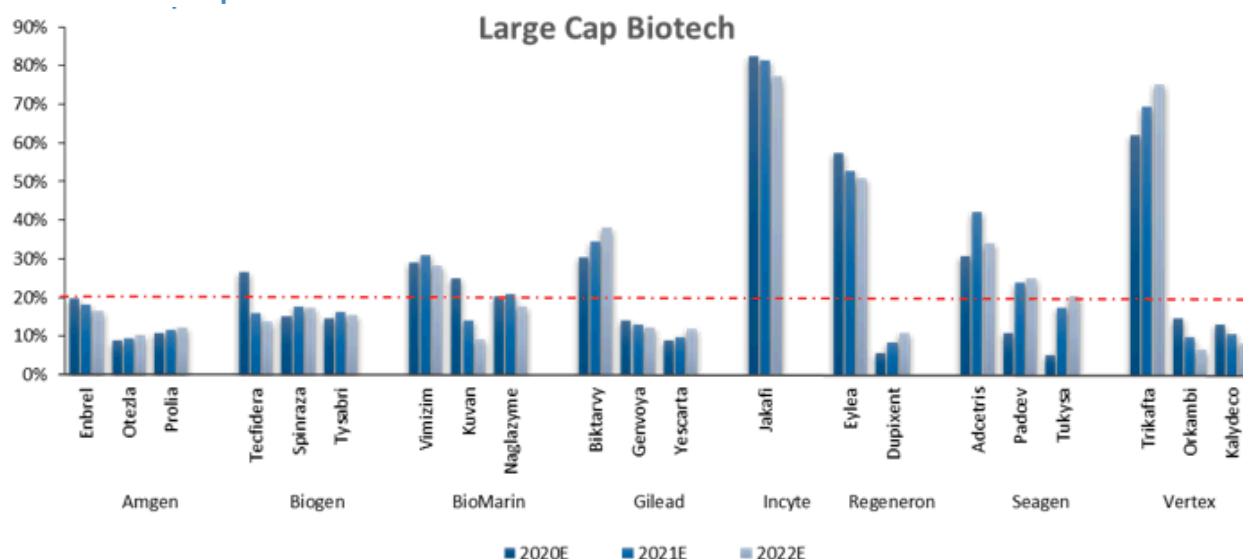
[#]Consensus exclusive to JZP-458 not available

^{###}Consensus estimates pulled from overall revenue estimate rather than product estimate

Key Themes for 2021: Concentration Risk

Assessing relative concentration risk across large-cap biotech

With the exception of AMGN, combined sales from top 3 selling products of biotech companies generally represent >50% of the total revenue, with few like INCY, REGN and VRTX depending solely on their top products (>50%), which underscores the need for M&A and business development



Key Themes for 2021: Uses of Cash

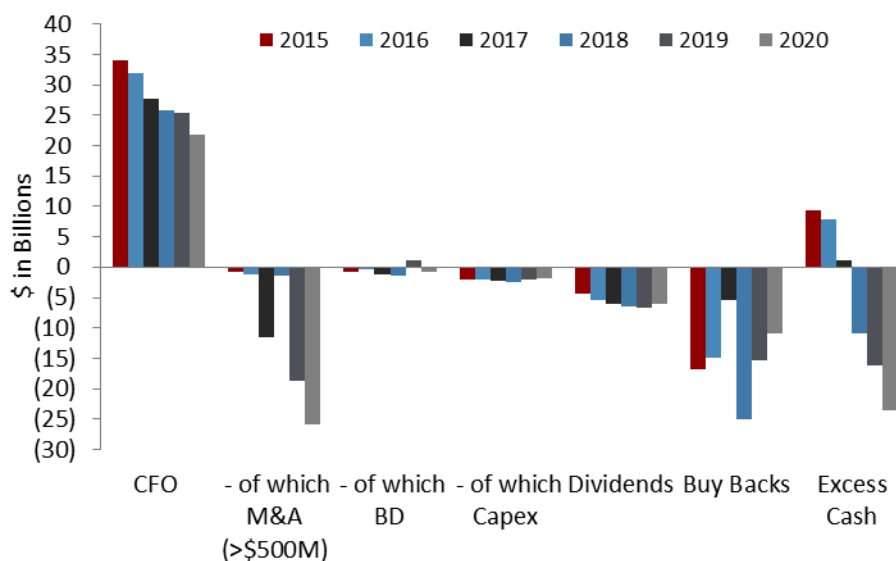
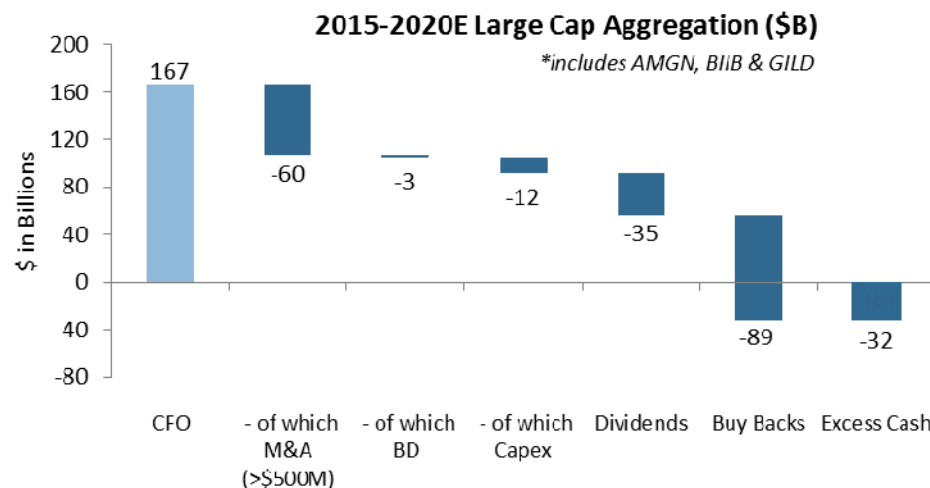
We expect continued focus on capital allocation in 2021

CF from operations expected to increase in 2021; we estimate ~\$25B in combined CFO for AMGN, BIIB & GILD

- GILD is expected to see an increase from \$7.1B in 2020 to an estimated \$11.1B in 2021 in cash flow from operations
- Capital allocation from 2015 to 2020E...
 - Share buybacks (\$89B)
 - M&A (\$60B)
 - Capital expenditures (\$12B)
 - Dividend (GILD and AMGN, \$35B)

We anticipate continued focus on M&A/BD as growth levers are drying up... although in the absence of attractive deals, companies may turn to accelerated share repurchase (as has often been the case in prior years)

- GILD and AMGN are the only dividend-issuing biotech companies, and we expect that will continue for the foreseeable future
- Given growing cash balances and concerns about long-term revenue growth, M&A continues to be an area of significant focus among the Large Caps
- AMGN, BIIB and GILD ended 3Q20 with \$12B, \$5B and \$26B* in cash, respectively



* GILD's cash as of 3Q20 doesn't take into account the Immunomedics acquisition

Key Themes for 2021: Uses of Cash / M&A

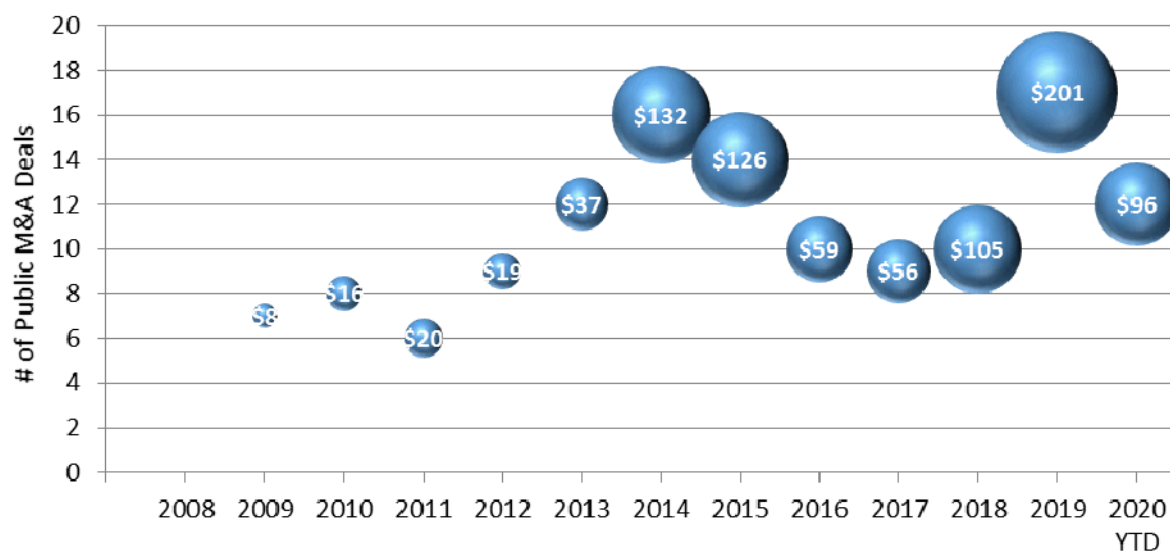
Capital deployed for M&A activity in 2020 was down quite a bit from all-time high levels in 2019

M&A deals in 2020 were significantly lower in both number and value than 2019, although we acknowledge that 2019 was a tough comp for M&A activity

- Through 12/15/2020, 12 public M&A deals were announced in the US vs. 17 in 2019 with a combined deal value of \$96B (vs. \$201B in 2019); historically, 2019 was the highest level of capital deployed by companies for public M&A activity
- Bulk of this activity was driven by one large-sized acquisition and a couple of mid-sized transactions (largest deal was AZN acquiring ALXN for \$39B)
- Small-sized bolt-ons kept materializing throughout the year; however, uncertainty due to the COVID-19 pandemic impacted the environment for M&A, and momentum never really picked up steam
- SMID-cap valuations could have also led to a bid/ask disconnect

While hard to predict, we think M&A activity will once again be a meaningful driver of sentiment in 2021

- With mounting cash balances and intensified need for near and longer term growth, we think large biopharma companies will continue to look to external innovation to augment pipelines and commercial portfolios; in many cases we believe this is an increasingly important issue for companies
- Essentially all of Large Cap biotech and Pharma have publicly indicated an interest in exploring M&A (of varying scope) throughout the year; some examples include GILD, PFE, MRK, LLY, NVS, JNJ, Roche, AMGN, BIIB, and SNY... among many others



* 2019 includes Amgen's acquisition of drug Otezla

Key Themes for 2021: Uses of Cash / M&A

M&A activity could center on assets in “hot” therapeutic areas

We expect focus to remain on “hot” areas with high strategic value (list not meant to be comprehensive)

■ Oncology / Hematology

- Commercial synergies are commonly highlighted in these transactions

■ Orphan disease

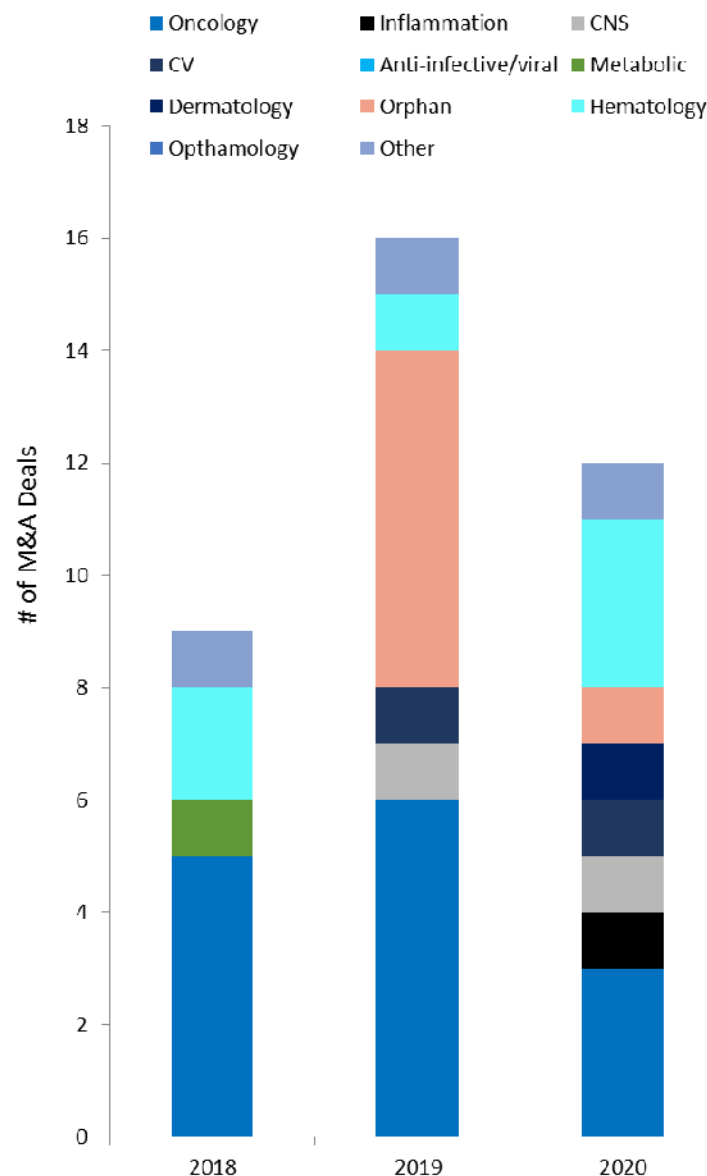
- Pricing power, regulatory designations, and other benefits (e.g., tax credits) continue to increase the relative value of orphan assets (although to a lesser extent in recent years)

■ CNS

- CNS has also been noted as a “hot area” given the unmet need and potentially large markets

We also think unencumbered assets with clear value propositions could be attractive targets in 2021

- One-product companies may represent opportunities for relatively easy integration
- Focus on agents that have potential in multiple indications has also been a more recent theme
- Further, there continues to be interest to acquire new capabilities / platforms



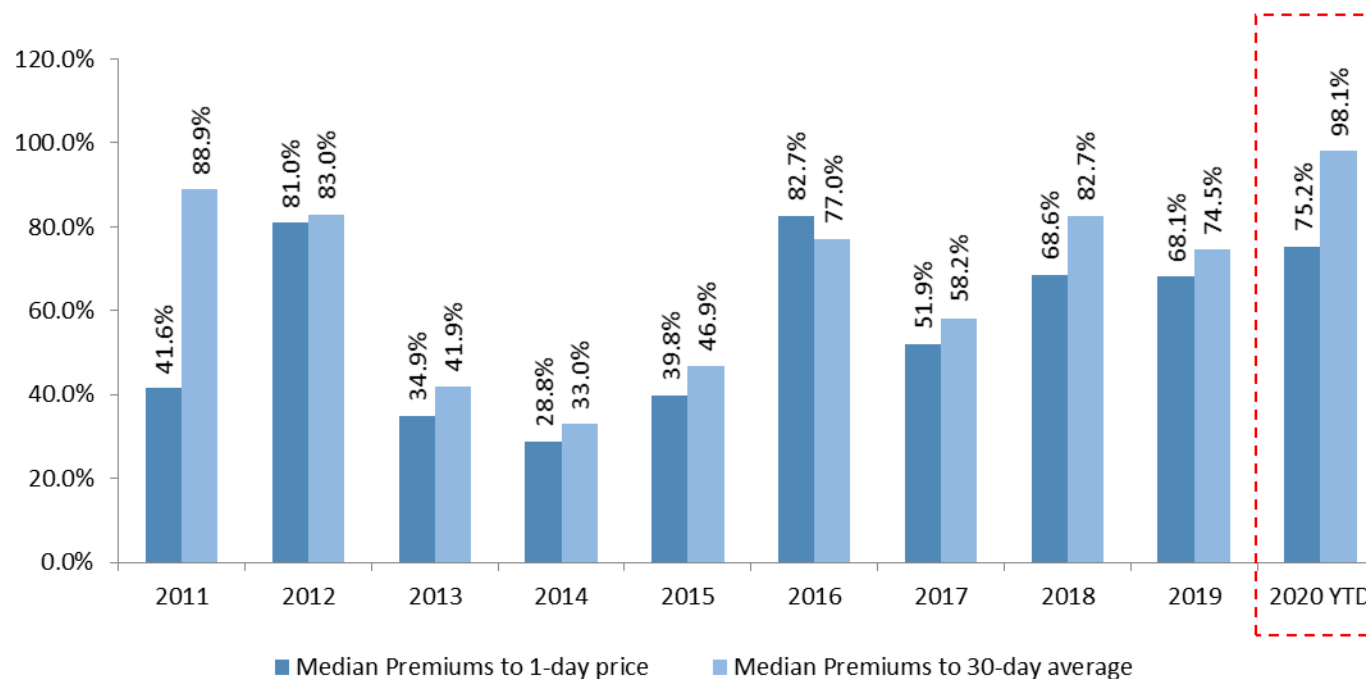
Key Themes for 2021: Uses of Cash / M&A

If there is an uptick in 2021 M&A, it could lead to upside across the SMid-cap space

We took a closer look at recent public M&A deals to get a sense of premiums over the last 10 years...

- Median premiums paid on the prior one-day price (for deals >\$500M) have been ~75% YTD (and ~98% to the 30-day average) compared with ~68% (~75% 30 day) in 2019 and ~69% (~83% 30 day) in 2018
- Average premiums in 2020 emerged as one of the highest in the last 10 years. Notably, the average premium paid has been on an uptrend in the last 3-4 years, with current premiums reaching up to ~83% in 2020 and ~76% in 2019
- Generally, larger deals (>\$20B) tend to have lower premiums in the 30-40% range, while smaller deals (<\$20B) tend to hover in the 50-70% range. In 2020, the single large-sized deal had a premium of 45% whereas for smaller deals the median premium stood at 80%

If deal momentum picks up in 2021, we could see meaningful upside across the SMid-cap space as potential M&A premiums could further work their way into valuations



Key Themes for 2021: Uses of Cash / M&A

Overall, the large-cap biotechs maintain significant financial flexibility to trigger M&A in 2021

We believe that the large-cap biotechs have enough fire-power – and need – to execute on meaningful M&A...

- Between a favorable mix of cash on hand (~\$37B*), FCF generation (we est. cumulative ~\$125B through 2024), and the ability to lever up the balance sheet (assuming anywhere from 2x to 4x net-debt/EBITDA), large caps are well positioned to execute on further business development (further boosted by the tailwind of lower effective US tax rates)

	Current Cash Position (\$B)	Cumulative FCF			Effective Tax Rate			
		2020E (1 Year)	2020E-22E (3-Years)	2020E-24E (5-Years)	2020E	2021E	2022E	2023E
AMGN	\$12B	\$10B	\$31B	\$53B	13.4%	12.8%	13.9%	13.5%
BIIB	\$5B	\$5B	\$13B	\$20B	18.0%	19.7%	20.5%	21.4%
GILD	\$11B	-\$15B	\$0B	\$16B	19.7%	16.9%	18.0%	18.8%
Total	\$28B	\$0B	\$44B	\$89B				
REGN	\$2B	\$2B	\$15B	\$22B	12.3%	13.6%	13.9%	13.6%
VRTX	\$6B	\$2B	\$8B	\$14B	20.6%	22.0%	21.8%	21.7%
Total	\$9B	\$5B	\$22B	\$36B				

	Current Cash Position* (\$B)	Current Debt Position (\$B)	JPMe EBITDA 2020E	Current Net Debt/ EBITDA 20E	Debt Capacity (2x)	Debt Capacity (3x)	Debt Capacity (4x)
AMGN	\$12B	\$34B	\$15B	1.5x	\$8B	\$23B	\$37B
BIIB	\$5B	\$8B	\$6B	0.5x	\$10B	\$16B	\$22B
GILD	\$11B	\$35B	\$4B	5.5x	-\$15B	-\$11B	-\$7B
Total	\$28B	\$77B	\$26B	1.5x	\$2B	\$27B	\$53B
REGN	\$2B	\$4B	\$4B	0.5x	\$6B	\$10B	\$14B
VRTX	\$6B	\$1B	\$3B	-1.9x	\$11B	\$14B	\$17B
Total	\$9B	\$5B	\$7B	-0.7x	\$17B	\$24B	\$31B

... and investors both expect and want to see more M&A in 2021

- In our Dec 2020 buy-side survey, 79% of responders expected an uptick in M&A in 2021 relative to 2020; this is much higher when compared with our Dec. 2019 survey (56%)

*Note that GILD's cash & debt position incorporates the \$15B cash paid and \$6B debt issued for the Immunomedics acquisition

Key Themes for 2021: Uses of Cash

Historically, R&D as a percentage of sales is not surprisingly higher for Biotechs relative to Pharma

Over the years, large-cap biotech has higher R&D expense as a proportion of sales relative to Pharma

- It is particularly high among BMRN, INCY, REGN, SGEN & VRTX, highlighting the capital-intensive nature of the sector

Within pharma, major pharma has a higher proportion of R&D as percent of sales compared with spec pharma, as anticipated

- Single-digit percentages indicate the lower R&D business models of the spec pharma companies
- AZN, BMY, LLY & Roche have higher spending than their peers

Also among major pharma, the proportions are similar between American and European companies

- Top 5 players range 17-25% and 14-25% in the US and EU, respectively

R&D as % of Sales		2013	2014	2015	2016	2017	2018	2019
US Biotech	ALXN	18%	16%	20%	22%	21%	16%	18%
	AMGN	22%	21%	19%	17%	16%	16%	18%
	BIIB	26%	23%	22%	20%	22%	24%	16%
	BMRN	61%	59%	66%	55%	44%	43%	42%
	GILD	18%	11%	9%	13%	13%	16%	41%
	INCY	111%	97%	80%	66%	73%	75%	53%
	REGN	52%	62%	51%	52%	48%	48%	39%
	SGEN	151%	130%	130%	143%	148%	118%	78%
	VRTX	100%	151%	84%	53%	53%	40%	42%
US Pharma	MRK	16%	15%	17%	17%	19%	19%	21%
	PFE	14%	16%	17%	16%	16%	16%	17%
	ABBV	15%	16%	16%	16%	17%	16%	19%
	LLY	26%	27%	29%	29%	27%	25%	25%
	BMY	40%	37%	30%	26%	26%	25%	24%
US Spec Pharma	VRX / BHC	3%	3%	3%	5%	5%	6%	5%
	MYL	7%	7%	7%	6%	6%	5%	NA
	TEVA	7%	7%	8%	8%	8%	6%	6%
	AGN	8%	9%	9%	10%	10%	10%	11%
EU Pharma	SNY	15%	15%	15%	16%	18%	20%	16%
	NVS	24%	24%	23%	21%	21%	20%	19%
	Roche	26%	24%	25%	25%	25%	27%	21%
	AZN	17%	19%	25%	26%	27%	25%	25%
	GSK	17%	17%	20%	18%	20%	18%	14%

GILD was lower in 2014/2015 due to HCV launches

Key Themes for 2021: Uses of Cash

Does increased R&D spend in the sector lead to higher value generation in the long run?

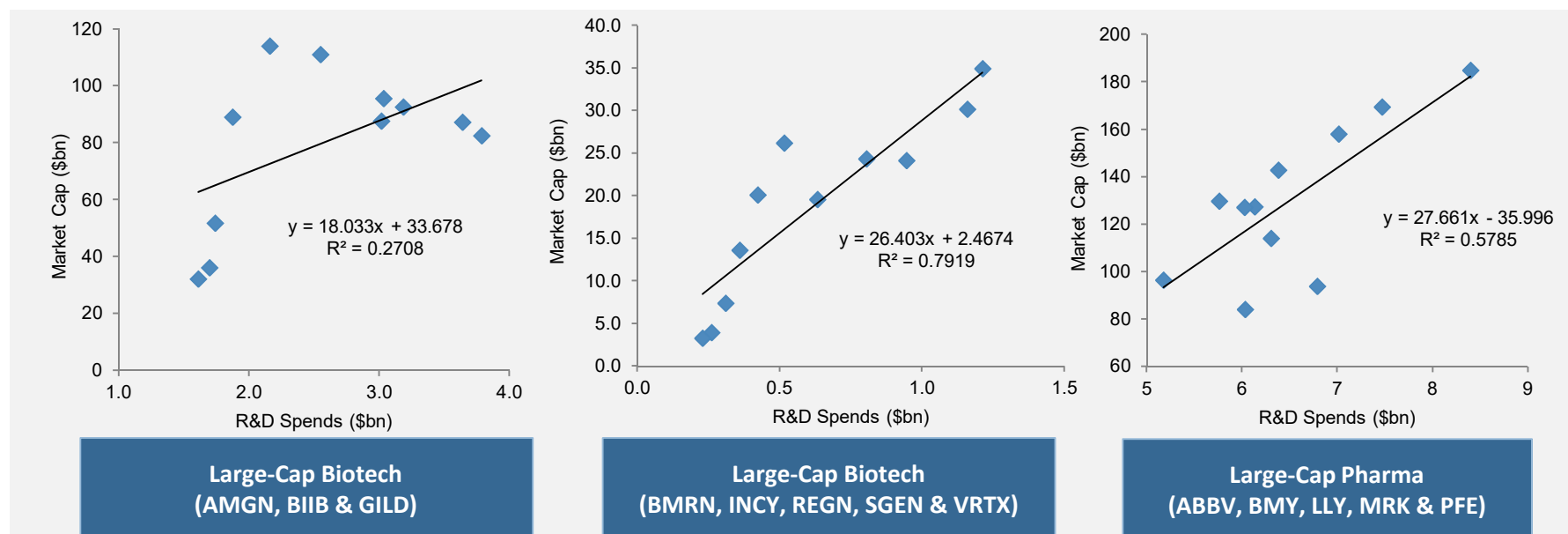
Over the last 10 years, mean R&D spend (GAAP) during any year has had a linear relationship with the average market value of the company after two years

- This relationship is higher in the case of Large-Cap Biotechs including BMRN, INCY, REGN, SGEN & VRTX (correlation of 0.89) followed by AMGN, BIIB & GILD (correlation of 0.52, emphasizing the significance of return generation due to pipeline investment)

Investment in biotech companies over the years in general hasn't returned as much relative to pharma

- R&D investment has resulted in significant value creation over the years; however, the translation is slightly lower in case of pharma (correlation of 0.76), potentially indicative of better returns from acquiring late-stage assets

Not surprisingly, SGEN & VRTX (both with correlation of >0.9) among the Large Cap Biotechs offer the highest benefit in terms of return due to the rapid revenue growth of newly launched products



*Average Market cap at the end of the year is regressed with average R&D spend two years prior

Key Themes for 2021: Capital Markets

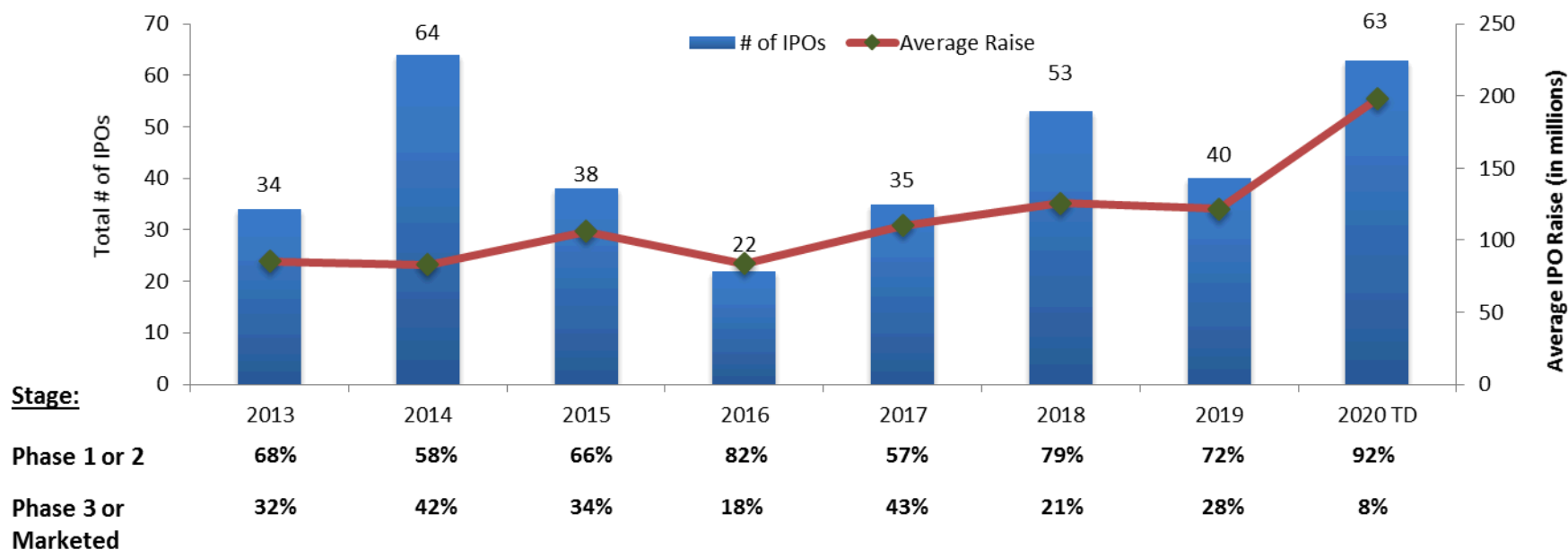
Capital markets remain healthy with a record high number of public offerings as well as capital raised

IPO activity flourished in 2020, the latter half of the year being more active than 1H

- There have been 63 biotech IPOs in 2020 to date, up from 40 in 2019; 25 deals in 1H20 and 38 in 2H20 (through 11/27). The total value raised of ~\$12.5B, as well as the amount raised per co (\$198M), reached all-time high levels in 2020; nearly 2x the previous peak of ~\$6.6B raised in 2018.
- Similar to 2018/19, we saw a high share of earlier-stage IPOs in 2020, with **92% of deals involving pre-clinical or Phase 1/2 assets vs. 72% in 2019 and 79% in 2018**; lately IPOs have skewed toward more early-stage assets with high risk/reward.
 - We believe early-stage companies can still get out as long as they have a novel platform and/or a seasoned mgmt team.
- Analogous to previous years, IPOs were largely focused on companies in “hot” therapeutic markets, such as oncology (e.g., LEGN, BDTX, RVMD, SQZ), orphan disease (e.g., DYN), and CNS (e.g., ANNX, PASG, ATHA) or with novel platforms (e.g., GBIO, ALVR).

We expect companies with platform technologies and differentiated approaches to remain in focus in 2021

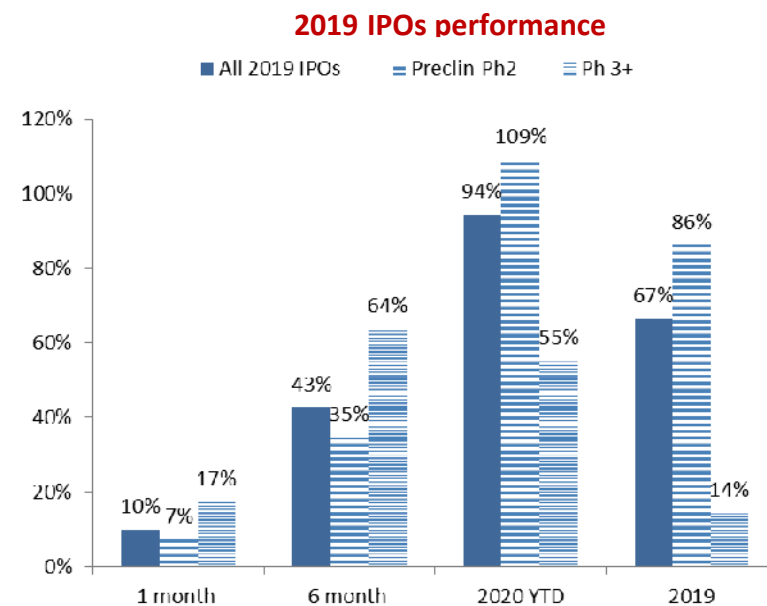
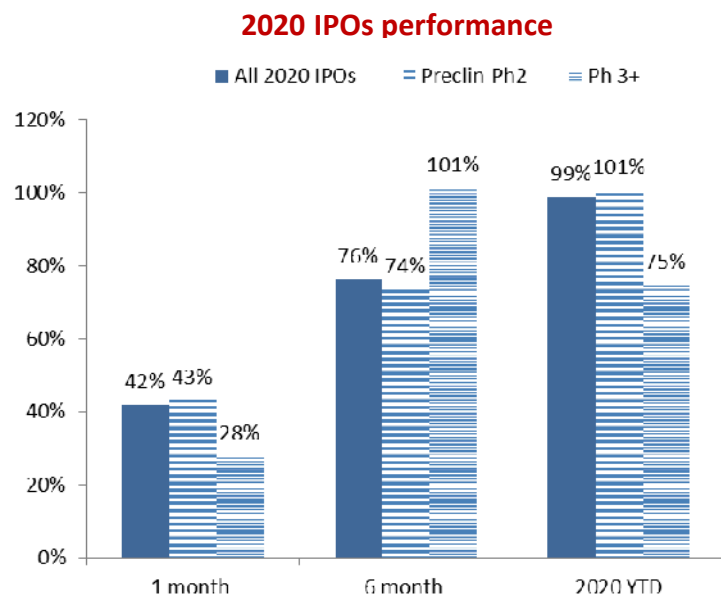
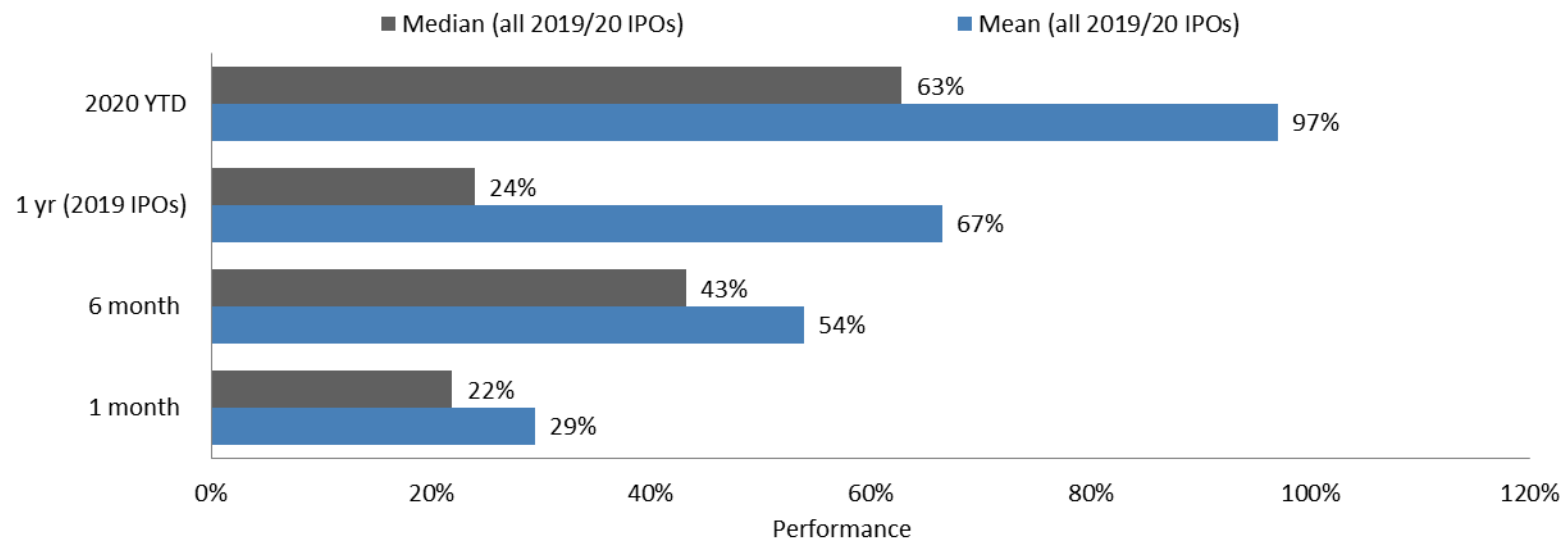
- Although this remains a topic of debate, we don’t see a closure to the proverbial IPO window forthcoming. While we continue to think that the market will be selective, we also believe companies with the right profile (compelling science, leadership teams, and development strategies) can get out... and obviously the healthier the market, the easier this will be.



Key Themes for 2021: Capital Markets

The IPO environment remains favorable, with very strong performances seen YTD

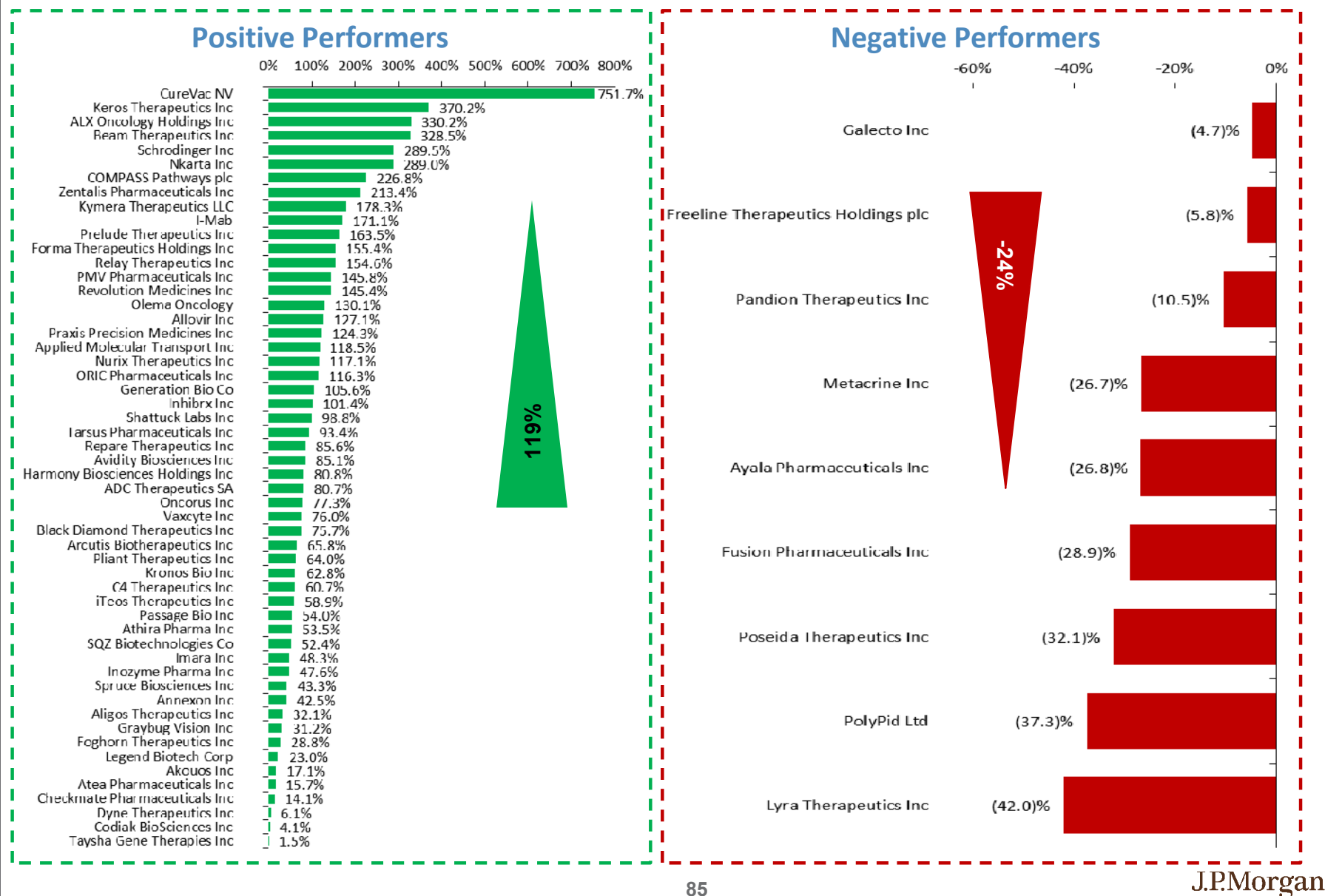
We continue to believe that there is an accommodating market for the right companies. On average, performance of new IPOs in 2020 and 2019 has been very strong year to date.



Key Themes for 2021: Capital Markets

On avg, IPOs in 2020 have strikingly outperformed the benchmark (99% vs. 23% for NBI), with 6x more positive performers than negative ones

IPOs performance in 2020 to date



Key 2021 Catalysts in Our Universe

Key Potential Catalysts Within Our Coverage – Kasimov

Top Kasimov catalysts to watch

Ticker	Catalyst	Timing	Comments
BMRN	Phase 3 topline data for Roctavian (1-year ABR)	early '21	Positive results are required for EU filing and could potentially de-risk the 2-year ABR data expected YE21/early '22 for US approval
BMRN	Phase 3 data for vosoritide at 2-years	Jan. 2021	We expect results to incrementally reduce regulatory risk ahead of the Aug. 20 PDUFA
AMGN	Phase 2 data for sotorasib/AMG 510	Jan. 2021	Full results from the positive ph2 NSCLC study are expected that will provide visibility on the competitive dynamic of the KRAS G12C space
LEGN	Complete submission of cilta-cel regulatory filing in r/r MM	Early '21	On the heels of the +ve ASH update, we expect JNJ/LEGN to file for approval shortly
BIIB	Aducanumab PDUFA	7-Mar-21	The PDUFA for aducanumab is 3/7/2021.
BLUE	The potential approval of lce-cel in r/r MM	27-Mar-21	The PDUFA for lce-cel is 3/27/2021. While we fully expect lce-cel to be an approval product, mgmt was cautious on whether this would occur on or before the PDUFA date
ACAD	Potential Nuplazid approval in DRP	3-Apr-21	The PDUFA in DRP is key to unlocking significant value from Nuplazid as the patient population is ~10x that of the currently approved indication of PDP
AMGN	Full Ph3 tezepelumab data in severe asthma	1H21	Following positive topline ph 3 data; the full analysis should increase visibility on teze's safety profile and commercial potential in a broad asthma population
AMGN	Sotorasib combo updates in solid tumors	1H21	Combo results will provide additional clarity on the commercial potential of sotorasib; including the potential utilization in the 1L NSCLC setting
BIIB / SAGE	Phase 3 zuranolone WATERFALL data (MDD)	1H21	WATERFALL is the first of three pivotal Phase 3 readouts for zuranolone and 2021, and looks to improve on the near-miss from the MOUNTAIN study
ALLO	Ongoing ALLO-501 updates and initial '501A results	1H21	We're cautious on the '501 update as this is expected in 1H21 (vs. being presented at ASH) and the company is moving to a consolidation-like approach
VRTX	Phase 2 VX-864 data in AATD	1H21	After VX-814 was discontinued, VRTX is down to its backup molecule, VX-864, to revive the AATD program.
MRNA / BNTX	Competitive COVID-19 vaccine updates	1H21	Although there isn't a single competitive update we're looking at, there are multiple Phase 3 trials ongoing and each update will inform the ultimate market opportunity for MRNA and BNTX
BMRN	Phase 1/2 data for Roctavian at 5-years	mid 2021	Results will provide additional visibility into the durability of Roctavian which will establish the clinical bar for competitive products and read-through to commercial expectations
GWPH	Phase 3 nabiximols data	mid-2021	Data from any one of the five Phase 3 US nabiximols studies could support registration
BMRN	Potential vosoritide approval	20-Aug-21	The approval for vosoritide could result in the first novel drug for the treatment of achondroplasia and partially restore the sentiment hit on the heels of the Roctavian CRL
GILD	Interim results for Trodelvy in HR+ BC	2H21	Results from this trial will inform the utility of Trodelvy in HR+ breast cancer
SGEN	Potential update for Padcev in 1st line bladder cancer from EV-103 cohort K	2H21	Data from EV-103 Cohort K will be used as the basis to file for accelerated approval in 1L bladder cancer (pt ineligible)
INCY	Phase 3 data from topical rux in vitiligo	2021	Vitlgo is the second indication INCY is pursuing for topical rux, potentially expanding the opportunity beyond atopic dermatitis
NVCR	Interim results for Phase 3 studies in solid tumors	2021	The company expects interim results from the Phase 3 LUNAR (NSCLC), INNOVATE-3 (Ovarian) and PANOVA-3 (pancreatic) studies; however, data are unlikely and the studies are expected to run to completion
GILD	Pivotal results with Yescarta in 2L DLBCL	2021	Full results from this study will inform the opportunity for Yescarta (and CAR T more generally) in earlier lines of DLBCL
BIIB / SAGE	Phase 3 zuranolone SKYLARK (PPD) and CORAL (RRT) data	2021	The additional two Phase 3 readouts for zuranolone are in PPD and RRT, expanding the drugs indications and breadth.
BMRN	Phase 3 data for Roctavian (2-year ABR)	YE21/early '22	Phase 3 data at 2-years are required for US registration

Key Potential Catalysts Within Our Coverage – Fye

Top Fye catalysts to watch

Ticker	Catalyst	Timing	Comments
AMRN	Potential EU approval for Vascepa	Early 2021	We see no EU exclusivity impact from US patent rulings, but expect slow roll outs by country and incr '21 contribution
BCRX	Potential approval of berotralstat in HAE in EU	Early 2021	Following US, we expect approval in Europe as well
EBS	Ph III data for COVID-HIG in adult patients hospitalized with COVID-19	Early 2021	This could potentially support an EUA application
IONS	Phase II data for IONIS-GHR-LRx in treatment-naïve acromegaly patients	Early 2021	Mgmt sees data from uncontrolled pts to helping guide reg discussions/design of the phase III
ZYME	Initial readout of phase I dose escalation study of ZW49	Early 2021	We expect a dose escalation update focused on higher doses and with some of the cohorts beyond 3 pts
ARNA	Ph I/II (CAPTIVATE) data for olorinab in abdominal pain assoc with IBS-C/D	1Q21	We see readout from CAPTIVATE as a call option not priced in the stock
BCRX	'9930 data for (up to 500mg) C5 inhib poor responders and naïve pts in PNH	1Q21	We see this update rounding out the profile of '9930 and helping investors to better understand the potential
DNLI	Phase I/II interim biomarker data demonstrating rescue of lysosomal function	1Q21	We expect biomarker data to show rescue of lysosome function and support the dose for late-stage development
KLDO	Topline KB109 data in non-IND studies (K031, K032) in mild-to-mod COVID-19	1Q21	We are cautious on the amt of value that could be ascribed for initial data as neither trial is powered for efficacy
ASND	Potential US approval (June 25 PDUFA)	2Q21	We see this approval and launch as the first conclusive delivery on the promise of the TransCon platform
UTHR	Potential approval of Tyvaso for PH-ILD	2Q21	We believe the PH-ILD population could represent a substantial opportunity for Tyvaso
IONS	Phase II data for IONIS-PKK-LRx in HAE	1H21	Watching for strong attack rate reduction which we think will be needed to be competitive + infrequent LICA dosing
IONS	Pulmonary delivery data for IONIS-ENAC-2.5Rx in cystic fibrosis pts	1H21	Potential to provide POC for IONIS-ENAC-2.5Rx in cystic fibrosis
IONS	15-month open label tominersen + 15-month natural history data	1H21	Expect Roche to present safety/tolerability, drops in huntingtin, and endpoints similar to those in GENERATION HD1
NKTR	Initial safety and ORR data from PROPEL study	1H21	We see this data potentially informing the development strategy in 1L NSCLC
RUBY	Interim data update from phase I dose escalation for RTX-240	1H21	Expect early look at RTX-240 immune stimulation, but pot'l anti-tumor activity could be needed for more stock credit
DNLI	Phase I/II final data demonstrating reduction in NF-L	Mid-2021	Expect this could inform the level of GAG reduction sufficient to treat neurologic manifestations of Hunter Syndrome
JAZZ	Potential JZP-458 launch following approval	Mid-2021	We see a good probability for JZP-458 to receive approval and largely replace/expand use beyond Erwinaze
KLDO	Topline data from study of KB295 in mild-to-moderate UC	Mid-2021	Mgmt is looking for topline results to show safety/tolerability and an impact on Simple Clinical Colitis Activity Index
IMGN	Topline ph III SORAYA data for mirv in post-Avastin PROC pts with high FRα	3Q21	We see a good POS for SORAYA, setting up a potential accelerated approval in 2022
JAZZ	Potential approval of Xywav in IH following sNDA submission in 1Q21	4Q21	We view the opportunity in IH as potentially underappreciated and see ~\$300mm+ peak revs
LXRX	Data readouts from phase II studies in DPN and PHN for LX9211	4Q21	Mgmt expects the phase II studies to provide POC for LX9211 in chronic pain
IONS	Dose-ranging data from TRANSLATE-TIMI 70 trial for vuparnorsen	2H21	Look for results to guide phase III study design
IONS	Topline data from phase III (VALOR) study of tofersen in SOD1-ALS patients	2H21	With an enriched pop (~60% rapid progressors), watching for stabilization of ALS
EBS	Potential decision on NARCAN appeal	2H21	We expect entry of generic naloxone in 2H21
ARNA	Phase II/III (CULTIVATE) dose-ranging data for etrasimod in Crohn's disease	2H21	We see these data informing the profile of the 2mg and 3mg doses in CD and reconfirming activity in GI
DCPH	Initial readout from phase III INTRIGUE trial in 2L GIST	2H21	With robust benefit in INVICTUS and ph I showing 10.7 mos mPFS in 2L GIST (n=31), we expect success for INTRIGUE
IONS	Phase I/II top-line data for IONIS-MAPTRx in Alzheimer's	2H21	Mgmt expects to see clearing or reduction of tau in patients before a later update on cognitive effects
IONS	Phase I/II data for IONIS-C9Rx in C9-ALS	2H21	Mgmt expects this data to establish a safety profile for IONIS-C9Rx in ALS patients
ITCI	Potential approval of Caplyta in bipolar depression	2H21	With two successful phase IIIs, we expect approval for depression in Bipolar I & II as mono- and adjunctive therapy
KLDO	Topline phase II KB195 data (UNLOCKED) in UCD inadequately controlled on SOC	2H21	We see this data as key to validate '195's ability to drive a compelling benefit in UCD and derisk the MMT platform
RDUS	Ph III EMERALD data for elacestrant in ER+/HER- adv MBC (licensed to Menarini)	2H21	We see a elacestrant economics as a cheap option in the stock
RDUS	Data from phase III wearABLE study	2H21	See patch offering the potential to expand the Tymlos comm'l opttty and extend the durability of the franchise
AMRN	Potential updates on suit vs Hikma re: US patents '537, '077, '861	2021	Interesting, but this legal path could be a reach, as we believe it could be difficult to prove Hikma induced off-label use
MGTA	Results from phase II studies of MGTA-145	2021	We expect data to reinforce mobilization of functional HSCs and, more importantly, demonstrate engraftment
MGTA	Initial clinical data for MGTA-117	2021	We see this initial data as key for investors to better assess the value proposition of this program
STOK	Data readout from SAD portion of the MONARCH study of STK-001 in Dravet	2021	While still unknown if these single doses will offer therapeutic exposure, we see PK key to inform when might see efficacy
UTHR	Potential launch of ISR and RemUnity	2021	We see launch of the pumps as key for the durability of the Remodulin franchise by insulating the biz from generics
RDUS	Potential approval of Tymlos in Japan (with partner Teijin)	2021	We see a good probability of success for the registrational filing of abaloparotide injection in Japan
NKTR	Pot'l first data from pivotal trial for bempeg in 1L metastatic melanoma	Late-2021/Early '22	Interim analysis remains based on ORR; we see low odds that this hits
ARNA	Phase III (ELEVATE) data for etrasimod in UC 12 and UC 52 trials	1Q22	We see a very high probability of success for the ELEVATE trials based on ph II OASIS data and read from ozanimod

Key Potential Catalysts Within Our Coverage – Rama

Top Rama catalysts to watch

Ticker	Catalyst	Timing	Comments
ALNY	Topline phase 3 HELIOS-A data for vutrisiran in ATTR-PN	Early 2021	Report topline phase 3 vutrisiran HELIOS-A data in hATTR amyloidosis patients with polyneuropathy (ATTR-PN)
FOLD	Initial phase 1/2 CLN3 gene therapy data in Batten Disease	Early 2021	Report initial data from the AAV9-CLN3 gene therapy program for CLN3-Batten Disease; regulatory interactions are ongoing and Amicus expects to provide the path forward in 2021
SRPT	Topline phase 2 Study-102 data for SRP-9001 in DMD	Early 2021	Report topline phase 2 placebo-controlled data in Duchenne Muscular Dystrophy (DMD), with the primary efficacy analysis at Week 48
IFRX	Phase 3 interim analysis data for IFX-1 in COVID-19 induced pneumonia	Early 2021	Report data (interim analysis) from the phase 3 portion of the phase 2/3 adaptive, randomized controlled trial (of IFX-1+ best supportive care (BSC) versus placebo + BSC) in patients with COVID-19 pneumonia (interim analysis planned after 180 patients enrolled, with the potential for an early stop for efficacy or futility)
ZLAB	Zejula inclusion on NRDL	Early 2021	NRDL negotiations will conclude by year-end 2020 with announcements shortly after
ANAB	Regulatory update for imsidolimab in GPP	Early 2021	Report results from EOP2 with the FDA on path forward
VIR	Topline phase 3 COMET-ICE data for VIR-7831 in COVID-19	Jan-21	Report phase 3 VIR antibody (VIR-7831) data for COVID-19
GTHX	Trilaciclib PDUFA in SCLC	Feb-21	Watching for FDA approval of trilaciclib as a myelopreservation agent in small cell lung cancer (SCLC); PDUFA of 2/15/21 (under Priority Review)
FOLD	Topline phase 3 PROPEL data for AT-GAA in Pompe Disease	1Q21	Report topline phase 3 data in Pompe disease (includes ERT-naïve and switch patients); rolling BLA completion anticipated in 1H21 on positive results
AGIO	Topline phase 3 ACTIVATE-T data for mitapivat in regularly transfused adult PKD	1Q21	Report topline phase 3 mitapivat ACTIVATE-T data in adults with pyruvate kinase deficiency (PKD) who are regularly transfused; anticipated filing for U.S. and E.U. regulatory approval in adults with PKD in 2021
ANAB	Topline phase 2 POPLAR data in PPP	1Q21	Report initial data from the phase 2 trial of imsidolimab in palmoplantar pustulosis (PPP)
IDRA	Topline phase 3 ILLUMINATE-301 data in advanced melanoma	1Q21	Report topline data from pivotal study of tilsotolimod in advanced melanoma
FREQ	Topline phase 2a data for FX-322 in acquired SNHL	Late-1Q21	Report topline 90-day phase 2a data for FX-322 (single and repeat doses) in acquired sensorineural hearing loss (SNHL), with the end-of-study readout to follow in late-2Q21 (at day 210)
KNSA	Rilonacept PDUFA in RP	Mar-21	Watching for FDA approval and subsequent launch of rilonacept in recurrent pericarditis (RP) in the U.S.; PDUFA of 3/21/21 (under Priority Review)
GTHX	Phase 2 data from rintodestrant expansion (combination with palbociclib) arm in ER+ HER2- BC	2Q21	Report phase 1/2a data from the expansion arm of rintodestrant (800mg) + palbociclib in ER+, HER2- breast cancer (BC)
ITOS	Phase 1 data for EOS-850 (A2AR) and EOS-448 (TIGIT) in solid tumors	1H21	Report expansion results for '850 and initial results for '448, both in solid tumors
ORIC	Topline interim phase 1 data for ORIC-101	1H21	Report topline interim data in either prostate cancer (+enzalutamide) or solid tumors (+nab-paclitaxel) (and from the other in 2H21)
APLS	Topline phase 3 PRINCE data for pegcetacoplan in treatment-naïve PNH	1H21	Report topline phase 3 pegcetacoplan (APL-2) PRINCE data in patients with treatment-naïve Paroxysmal Nocturnal Hemoglobinuria (PNH)
APLS	Pegcetacoplan PDUFA in PNH	May-21	Watching for FDA approval of pegcetacoplan for the treatment of Paroxysmal Nocturnal Hemoglobinuria (PNH) in the U.S.; PDUFA of 5/14/21 (under Priority Review), with the approval in E.U. expected in 2021
SWTX	Topline phase 3 DeFi data for nirogacestat in desmoid tumors	2Q-3Q21	Report topline data from the potentially registrational DeFi trial in desmoid tumors
AGLE	Topline phase 3 pegzilarginase PEACE data in ARG1-D	Mid-2021	Report topline phase 3 data for pegzilarginase in Arginase 1 Deficiency (ARG1-D)
PASG	Initial phase 1/2 PGBM01 data in infantile GM1	Mid-2021	Report initial 30-day safety / biomarker data for the phase 1/2 trial for PGBM01 for the treatment of patients with infantile GM1 Gangliosidosis (GM1)
CCXI	Avacopan PDUFA in AAV	July-21	Watching for FDA approval and the potential launch of avacopan in ANCA-associated vasculitis (AAV) in the U.S. (PDUFA of 7/7/2021; approval in E.U. is expected in 2H21)
APLS	Topline phase 3 DERBY & OAKS data for pegcetacoplan in GA	3Q21	Report topline phase 3 pegcetacoplan DERBY & OAKS data in geographic atrophy (GA), with primary efficacy analysis at month 12
TBPH	Topline phase 3 SEQUOIA data for ampreloxtine in nOH	3Q21	Report topline results from the phase 3, four-week study for efficacy in nOH
YMAB	Approval of Omblastys in CNS/LM from NB	2H21	Re-file of the BLA submission is expected by late-2020 / early-2021; hence, watching for FDA (and EU) approval of Omblastys for the treatment of pediatric patients with CNS/leptomeningeal metastasis from neuroblastoma (CNS/LM in NB)
ANNX	Topline phase 2 data for ANX005 in HD	2H21	Report initial phase 2 data in patients with Huntington's Disease (HD)
ANNX	Topline phase 2 data for ANX005 in ALS	2H21	Report initial phase 2 data in patients with amyotrophic lateral sclerosis (ALS)
MRTX	File NDA for adagrasib (MRTX849)	2H21	Potential NDA filing for adagrasib monotherapy in KRASG12Cm 2L+ NSCLC
ALVR	Initial POC prevention data for Viralym-M	2021	Report initial proof-of-concept (POC) prevention data in 5 viruses and solid organ transplant (SOT)
IDYA	Topline interim phase 1/2 IDE196 data	2021	Interim IDE196 monotherapy data from the phase 1/2 GNAQ/11 basket trial
BBIO	Phase 3 acoramidis data from ATTRIBUTE-CM	Late '21 / early '22	Initial data (functional outcomes) from ATTRIBUTE-CM

RIGL is covered by J.P. Morgan Analyst Tessa T Romero;
IDRA is covered by J.P. Morgan Analyst Matthew Bannon

Key Potential Catalysts Within Our Coverage – Joseph

Top Joseph catalysts to watch

Ticker	Catalyst	Timing	Comments
XLRN	Topline BEYOND phase 2 data for luspatercept in non-transfusion dependent β -Thalassemia	4Q20/1Q21	Positive Hb data would de-risk expanded commercial opportunity in NTD patients. Registration enabling potential of BEYOND dependent on strength of results
AKBA	Vadadustat NDA submission for dialysis and non-dialysis CKD settings	1Q21	Recall, mixed MACE outcomes across the phase 3 program; positive in INNO2VATE (DD-CKD anemia) and negative in ITT population of PRO2TECT (NDD-CKD anemia)
KPTI	XPOVIO® potential label expansion BOSTON PDUFA (March 19th)	1Q21	Anticipating approval of XPOVIO in earlier-line Multiple Myeloma treatment setting
KPTI	NDA submission for XPOVIO® in liposarcoma	1Q21	NDA supported by top-line results from phase 3 SEAL study in 2L/chemo-refractory liposarcoma
MYOV	SPIRIT extension (1yr safety/efficacy data in endometriosis)	1Q21	12-month safety / efficacy results expected to support NDA/MAA submission and commercialization
NVAX	NVX-CoV2373: Phase 3 UK interim data	1Q21	Initial vaccine efficacy for NVAX COVID-19 vaccine candidate; anticipating readthrough to phase 3 US/Mexico trial and registration enabling potential in the US
TGTX	Regulatory action for umbralisib in MZL (2/15 PDUFA)	1Q21	NDA submission supported by Phase 3 UNITY-NHL study
ENTA	EDP-514 in HBV, preliminary data from phase 1b study in viremic patients; initial data (primarily safety) from phase 1b study in NUC-suppressed patients	2Q21	NUC-suppressed trial primarily assessing safety, tolerability; viremic study assessing safety, PK and antiviral activity in untreated patients with high viral load
ENTA	Data for EDP-297 (FXR agonist) phase 1 study	2Q21	Along with Phase 2 ARGON-2 study of EDP-305 will inform next steps for NASH program
MYOV	Regulatory decision for relugolix in UF (6/1 PDUFA)	2Q21	NDA submission supported by Phase 3
TGTX	Regulatory action for umbralisib in FL (6/15 PDUFA)	2Q21	NDA submission supported by the Phase 3 UNITY-NHL study
TGTX	BLA completion for U2 in CLL	1H21	Rolling BLA initiated December 2020; supported by Phase 3 UNITY-CLL study
AKRO	BALANCED Cohort C topline data (F4/cirrhotic NASH)	1H21	Evaluating 50mg QW EFX in 30 cirrhotic/F4 NASH subjects, 2:1 randomization vs pbo; will provide clarity on commercial opportunity beyond F1-F3
ALGS	ALG-010133 (STOPS) Phase 1a SAD/MAD data in HV	1H21	First in-human trials will assess safety, PK, and PD; subcutaneous dosing once every 7 days
ALGS	ALG-000184 (CAM) Phase 1a SAD/MAD data in HV	1H21	First in-human trials will assess safety, PK, and PD; once daily oral tablets
AVIR	AT-527 Phase 2 top-line data in mild/moderate COVID-19	1H21	Primary endpoint is reduction in progressive respiratory insufficiency compared to SOC
AVIR	AT-752 phase 1 HV trial initiation + completion	1H21	Primary endpoints include safety and PK at various doses
BDTX	Initial data from phase 1 dose-escalation portion of MasterKey-01 study of BDTX-189 (allosteric ErbB mutations)	1H21	Results expected to inform R2PD for MasterKey-01 study
MYOV	EU/MAA submission of relugolix for APC	1H21	MAA submission supported by phase 3 HERO study
MYOV	US/NDA submission of relugolix for endometriosis	1H21	NDA submission supported by phase 3 SPIRIT 1 & SPIRIT 2 studies
OBSV	US/NDA for linzagolix in UF	1H21	NDA submission on the basis of phase 3 PRIMROSE-1 & 2 studies
RCKT	Updated phase 2 data for RP-L201 in LAD-1	1H21	Anticipating efficacy data on CD18+ neutrophils counts; endpoint data evaluating patient survival at 2 years of age could enable regulatory filings
RCKT	Updated results from "Process B" study of RP-L102 in Fanconi Anemia	1H21	Results expected to include >12-month safety efficacy results from the phase 1 portion and preliminary safety and efficacy from the phase 2 portion
RVMD	Nominate 2nd RAS(ON) development candidate	1H21	Five RAS(ON) variant inhibitors currently in lead optimization; first development candidate expected by YE20 with 2nd candidate expected 1H21
RVMD	RMC-4630 monotherapy data (SHP2) in mixed solid tumors	1H21	Ongoing safety and efficacy data from intermittent schedule in the phase 1 trial
XLRN	Topline results from PULSAR OLE (sotatercept for PAH)	1H21	18-month follow-up results for eligible patients from PULSAR main study; expected to inform long-term benefit in phase 3 STELLAR pivotal trial
ENTA	12-week interim analysis of ARGON 2 (EDP-305) phase 2b in NASH	mid-2021	Along with EDP-297 phase 1 data will inform next steps of the NASH program including partnering potential with non-FXR mechanisms

Key Conferences Within Our Coverage

Key 2021 Medical Conferences

Meeting	Event (click for conference/association webpage)	Start Date	End Date	Location
ASCO-GI	ASCO Gastrointestinal Cancers Symposium	1/15/2021	1/17/2021	San Francisco, California
IASLC	International Association for the study of Lung Cancer	1/28/2021	1/31/2021	Singapore
ACAAI	American College of Allergy, Asthma and Immunology Meeting	2/26/2021	3/1/2021	San Diego, California
ECFS	European Cystic Fibrosis Society Conference	3/17/2021	3/21/2021	Albufeira, Portugal
ARVO	The Association for Research in Vision and Ophthalmology	5/2/2021	5/6/2021	San Francisco, California
ASGCT	American Society of Gene & Cell Therapy	5/12/2021	5/15/2021	Portland, OR
ATS	American Thoracic Society	5/14/2021	5/19/2021	San Diego, California
DDW	Digestive Disease Week	5/22/2021	5/25/2021	Washington, DC
EULAR	European Congress of Rheumatology	6/2/2021	6/5/2021	Paris, France
EPNS	European Paediatric Neurology Society	6/1/2021	6/5/2021	Glasgow, UK
ASCO	American Society of Clinical Oncology	6/4/2021	6/8/2021	Chicago, Illinois
EAACI	European Academy of Allergy and Clinical Immunology Congress	7/10/2021	7/12/2021	Madrid - Krakow
AHS	American Headache Society Scientific Meeting	TBD	TBD	
EHA	European Hematology Association	6/10/2021	6/13/2021	Vienna, Austria
ISTH	International Society of Thrombosis and Haematology	7/17/2021	7/21/2021	Philadelphia, Pennsylvania
IAS	International AIDS Society Conference	7/18/2021	7/21/2021	Berlin, Germany
EAN	European Academy of Neurology Congress	6/19/2021	6/22/2021	Vienna, Austria
CHEST	American College of Chest Physician's CHEST	6/24/2021	6/26/2021	Bologna, Italy
ADA	American Diabetes Association	6/25/2021	6/29/2021	Washington, DC
Cure SMA	Cure SMA Annual Conference	TBD	TBD	TBD
IAC	International Academy of Cardiology Meetings	TBD	TBD	TBD
AAIC	Alzheimer's Association International Conference	TBD	TBD	TBD
ESC	European Society of Cardiology	8/28/2021	9/1/2021	London, UK
ERS	European Respiratory Society International Congress	9/4/2021	9/8/2021	Barcelona, Spain
HFSA	Heart Failure Society of America	9/10/2021	9/13/2021	Aurora, Colorado
ESMO	European Society for Medical Oncology	9/17/2021	9/21/2021	Paris, France
WMS	World Muscle Society	9/21/2021	9/25/2021	Prague, Czech Republic
NACFC	North American Cystic Fibrosis Conference	9/30/2021	10/2/2021	San Antonio, Texas
ASBMR	American Society of Bone and Mineral Research	10/1/2021	10/4/2021	Toronto, Canada
ECTRIMS	European Committee for Treatment and Research in MS	10/13/2021	10/15/2021	Vienna, Austria
ANA	American Neurological Association	10/17/2021	10/19/2021	Washington, DC
ASN	American Society of Nephrology & Kidney Week	11/2/2021	11/7/2021	San Diego, California
ACR	American College of Rheumatology	11/5/2021	11/9/2021	San Francisco, California
ESGCT	European Society of Gene and Cell Therapy Congress	TBD	TBD	TBD
CTAD	Clinical Trials on Alzheimer's Disease	TBD	TBD	TBD
SITC	Society for Immunotherapy of Cancer	11/10/2021	11/14/2021	Washington, DC
AAO	American Academy of Ophthalmology	11/13/2021	11/16/2021	New Orleans, Louisiana
AHA	American Heart Association	11/13/2021	11/15/2021	Boston, MA
AASLD	American Academy for the Study of Liver Disease "The Liver Meeting"	TBD	TBD	
ASH	American Society of Hematology	TBD	TBD	San Diego, California
SABCS	San Antonio Breast Cancer Symposium	12/7/2021	12/11/2021	San Antonio, Texas
ESMO IO	European Society of Medical Oncology Immuno Oncology	TBD	TBD	Geneva, Switzerland

Favorite Names in 2021

Investment Ideas Across Various Themes

Beyond the J.P. Morgan team's top picks, we highlight additional names across a variety of investment themes...

	Kasimov	Fye	Rama	Joseph
Top pick(s)	BioMarin (BMRN)	Ascendis (ASND)	Sarepta (SRPT); Amicus (FOLD)	TG Therapeutics (TGTX)
Growth	Vertex (VRTX)	Jazz (JAZZ)	Apellis (APLS)	PTC Therapeutics (PTCT)
Value	X	United Therapeutics (UTHR)	Theravance (TBPH)	Accelaron (XLRN)
Event Driven	Sage (SAGE)	Zymeworks (ZYME)	SpringWorks (SWTX) AlloVir (ALVR)	Black Diamond (BDTX)
Launch Story	Acadia (ACAD)	BioCryst (BCRX)	Zai Lab (ZLAB)	Karyopharm (KPTI)
Turnaround	Legend Biotech (LEGN)	Rubius (RUBY)	AnaptysBio (ANAB)	Five Prime (FPRX)

Favorite Names: BioMarin Pharmaceuticals – Cory Kasimov

BioMarin (BMRN) – Overweight, Dec 2021 PT \$131

After a major regulatory setback, BMRN shares are attractive and have the potential to break out in 2021 as multiple clinical & regulatory catalysts approach...

Two clinical catalysts in the first few weeks of 2021 possibly hold the keys to investors wanting to get involved in 2021

- Both events have favorable risk-reward and could enable incremental de-risking for vosoritide and especially Roctavian
- Key catalysts anticipated for both products:
 - 1-year Ph3 top-line data for Roctavian in severe Hemophilia A are expected in early January (we suspect at the J.P. Morgan Healthcare Conference)
 - For vosoritide, Ph3 data at 2 years are expected in January, with potential approval by the August 20, 2021 PDUFA
- We believe that each product holds blockbuster potential
 - We estimate that vosoritide can eclipse \$1B in sales by 2026 (peaking at ~\$1.2B)
 - We acknowledge that there are likely far wider error bars for Roctavian; at this point we (potentially conservatively) project peak sales of ~\$1.7B (price is a key unknown, but we believe we are conservative)
 - To put in perspective, BMRN's current commercial portfolio (of 7 approved products) is expected to generate ~\$1.8B and ~\$2.0B in 2020 and 2021, respectively (7% CAGR 2020-25)

Bigger picture, BMRN looks attractively positioned with both a strong pipeline/R&D engine and a base business contributing ~\$2B

- While BMRN underperformed in 2020 (-8% YTD vs. NBI +24%), we believe shares can benefit from a strong growth outlook (we project a ~21% 2020-25 CAGR), better expense management, and especially its attractive late-stage pipeline/new product cycle
- On a DCF basis, we derive a value of \$131/share
 - This contemplates only 60%/70% probability of success for Roctavian and vosoritide, respectively, which we believe is conservative
- The value of the base business has been debated, which we value at \$75/sh; thus investors are not currently giving these programs much/any credit; updates from the earlier-stage pipeline (e.g., BMN 307/331 gene therapy for PKU/HAE) could also provide opportunity for share appreciation
- We believe BMRN carries significant longer-term scarcity value
- BMRN has ample cash (\$1.8B as of end-3Q20) to support its commercial/R&D operations

Where we could be wrong... If BMRN's pipeline encounters additional setbacks or the vosoritide filing hits a roadblock (e.g., manufacturing issue, regulatory pushback), and/or product sales fall short of expectations.

Other Investment Themes – Cory Kasimov

If growth is what you're looking for...

- **Vertex (VRTX):** With the launch of Trikafta under way, we think VRTX may be hard to ignore (for both specialists and generalists alike) given it's 5-year revenue and EPS growth profile (18% and 25% 5-year CAGR) relative to companies in the S&P index combined with intriguing pipeline optionality in 2020

For investors that are seeking value...

- **X**

If you're looking for an event driven name...

- **Sage Therapeutics (SAGE):** 2021 is nothing short of catalyst rich for SAGE with (1) zuranolone Phase 3 WATERFALL (MDD) data in 1H21; (2) Ph3 SKYLARK (PPD) in 2021; (3) Ph3 CORAL (RRT) in 2021; (4) randomized Phase 2 data from SAGE-324 in essential tremor in 1Q; and (5) Phase 2a data from SAGE-718 in Parkinson's Disease in 1Q21... amongst others. We acknowledge some of the upside potential has been capped in the near term on the BiIB collaboration, but we continue to believe that risk/reward is skewed higher.

Or a launch story....

- **Acadia (ACAD):** On the heels of Nuplazid gaining traction in the PDP market, ACAD's growth outlook has improved significantly following the positive Phase 3 DRP readout & filing (with a market size 10x the size of PDP). With Nuplazid's DRP PDUFA approaching (April 3rd), we highlight ACAD as a 2021 launch story that should garner investor attention as the sNDA has favorable short- and, especially, long-term implications for the company.

For investors who are seeking a turnaround story...

- **Legend Biotech (LEGN):** Following a bizarre string of mgmt departures ahead of positive results from cilta-cel (BCMA CAR T) at ASH 2020 (though not without some new safety considerations), we see LEGN as set up to be a turnaround story in 2021. Although we can appreciate some reluctance to own LEGN when there is some uncertainty in the nature of the parent company's issues, we can't ignore LEGN's valuation when considering that cilta-cel appears to be a best-in-class BCMA CAR T and better than other modalities that target BCMA too.

Favorite Names: Ascendis Pharma – Jessica Fye

Ascendis Pharma (ASND) – Overweight, Dec 2021 PT \$177

In 2020, ASND built on the success established with TransCon hGH with multiple positive data sets for TransCon PTH that reinforced the company's ability to enhance the profile of parent molecules. Looking ahead, we see a compelling case for continued steady value creation from additional products coming out of the platform including TransCon CNP in endocrinology as well as the emerging pipeline of oncology products all while ASND adds commercial revenue.

As Ascendis transitions to a commercial-stage company...

- We are optimistic about the uptake for TransCon hGH following its June PDUFA with a particular focus on those new to growth hormone treatments given the inherent churn in the market

... we see the pipeline advancing with a phase III start (PTH), a potential ph II readout (CNP), and two oncology candidates entering the clinic with potential for initial data from the first before year-end.

- The phase III trial for TransCon PTH is set to begin by early 2021, and we expect quick enrollment with pivotal data by YE 2022
- We continue to see TransCon CNP differentiated from vosoritide and see potential for data from the phase II study in 2021
- We expect initial clinical data for its first oncology candidate (TransCon TLR7/8 Agonist) by YE21 and IND/equivalent filing for TransCon IL-2 β / γ in 3Q21

Looking ahead, we see ASND poised to deliver continued value-enhancing updates from the pipeline, reinforcing the platform's potential while de-risking individual product opportunities.

Key Risks: Risks to our thesis/price target include negative regulatory outcome for TransCon hGH, or any negative clinical data for the company's other programs

Other Investment Themes – Jessica Fye

If growth is what you're looking for...

- **Jazz (JAZZ):** We see 2021 as a pivotal year for Jazz as it represents the first opportunity to firmly establish the durability of the oxybate franchise through the launch of Xywav, and we expect multiple expansion as this plays out. The Xywav switch (and IH oppty) are not the sole focus in 2021, however, as we also expect new oncology launches (Zepzelca, '458) to drive growth and diversify the company over time. Bigger picture, we see an attractive entry point in JAZZ shares with the stock trading at **11.4x** our 2021E EPS and anticipate multiple expansion as the company executes on the long-awaited transition to Xywav and its other new launches.

For investors who are seeking value...

- **United Therapeutics (UTHR):** While the US Remodulin business continued to hold up in 2020, in 2021 we are focused on UT's ability to launch the Remunity and ISR pumps and convert the franchise. We view such progress as key to not only insulate the business, but also for the stock to get credit for the business being durable. Beyond Remodulin, we see Tyvaso well on its way to becoming UT's largest product well ahead of the PH-ILD approval and are watching for the potential for TreT to further extend/expand that business. Net-net, we see both opportunities for upside to estimates and multiple expansion and a favorable outlook for UTHR stock.

If you're looking for a name exposed to a key event...

- **Zymeworks (ZYME):** We expect dose escalation data from the phase I study of ZW49 in early 2021 focused on clinically relevant higher doses, and these data are expected to underpin the rationale for mgmt's development decisions (although details will be preserved for presentation at a medical conference). We see these data as key for investors to better understand the asset's benefit/risk profile, in addition to setting the stage for eventual expansion cohorts, although we see anticipation built in to the share price and in general we view the stock as fairly valued at current levels.

Or a launch story....

- **BioCryst (BCRX):** On the back of US approval with a clean label and upcoming approvals in Japan and Europe, we see the Orladeyo launch in hereditary angioedema (HAE) attack prophylaxis as a key driver of stock performance going forward. Couple this with additional POC data for '9930 and potential move into later-stage trials and we see an attractive outlook for BCRX shares.

For investors who are seeking a turnaround story...

- **Rubius (RUBY):** We expect initial data from RTX-240 to provide early evidence of the product's ability to stimulate the immune system in the form of PD markers. If the update is accompanied by demonstrated anti-tumor activity, we think potentially substantial additional value could come into the stock as the market contemplates the potential for RTX-240 and the RCT platform more broadly.

Favorite Names: Sarepta – Anupam Rama

Sarepta Therapeutics (SRPT) – Overweight, Dec 2021 PT \$185

Phase 2 Study-102 data for lead asset SRP-9001 (micro-dystrophin gene therapy) in Duchenne Muscular Dystrophy (DMD) are expected in early 2021 and is a potentially transformative catalyst for the company

We continue to believe Study-102 has a high probability of success (see our comprehensive June 2020 slide deck [here](#))

- **We believe the efficacy benchmarks for a win/homerun scenario are achievable, based on the totality of prior phase 1/2 data**
 - Recall, the NSAA endpoint is the key clinical endpoint; of note, physician feedback suggests that ~1-3 point relative change (versus placebo) is supportive of a win (with the higher end of the range considered to be a homerun)
 - This seems achievable, given the ~5-6 point absolute benefit in the phase 1/2, with 3/4 patients achieving ≥5 point NSAA benefit at 12 months
 - We continue to believe the most conservative way to think about placebo is a ~1-2 point net benefit (though the company has suggested flat to down)
- **We also continue to believe that safety is a clear differentiation point for SRP-9001 relative to the competitive landscape of micro-/mini-dystrophin gene therapies (Pfizer/Solid); SRP-9001 has shown no complement-related AEs**

Positive Study-102 data could be the catalyst for credit for the broader pipeline

- **On positive Study-102 results, we see SRPT shares trading to ~\$200-225/share+ (versus down to the ~\$50-70/share range on a trial failure)**
 - Recent upside has shifted reward/risk but does not change our view on a positive outcome

Where we could be wrong... In our view, the biggest risk for SRPT shares centers around a clinical setback for Study-102 in the form of trial failure; in our view, one possible risk to Study-102 is potentially the heterogeneity of the progression of the patients enrolled positively impacting placebo performance

	SRP-9001 Study-102 Outcome	JPM Likelihood	JPM Model POS	SRP-9001 (Peak Revenues To SRPT)	Upside / Downside	Comments
JPM Current Base Model	-	-	80%	~\$3-4B	-	-
Scenario #1 (Homerun)	~2.5-3+ point relative benefit on NSAA Supportive secondary endpoints No major safety concerns (complement or otherwise)	40%	~90-100%	~\$3-4B	Higher end ~\$200-225/share	- Physician feedback suggest a NSAA relative benefit closer to ~3 points or better is a homerun scenario - Likely leads to POS increase and / or faster ramp / penetration - Potential for further sentiment based upside
Scenario #2 (Win)	~1-2.4+ point relative benefit on NSAA (closer to higher end of NSAA score) Supportive secondary endpoints No major safety concerns (complement or otherwise)	40%	~90-100%	~\$3-4B	Lower end ~\$200-225/share	- Physician feedback suggest a NSAA relative benefit in the ~1-2+ point range is considered clinically meaningful (though closer to 2 points or better would be a clear win) - Likely leads to POS increase - Potential for further sentiment based upside
Scenario #3 (Downside)	<1 point relative benefit on NSAA Discordant secondary endpoints Emergence of a unexpected safety signal	20%	0%	-	~\$50-70/share	- Disaster Scenario - Potentially removes entire SRP-9001 opportunity from the model - Value largely comes from exon skipping products, LGMD2E, platform optionality (though questions would emerge across the board) - Potential for further sentiment based downside

Favorite Names: Amicus – Anupam Rama

Amicus Therapeutics (FOLD) – Overweight, Dec 2021 PT \$29

Phase 3 PROPEL data for AT-GAA (ABT200 ERT + AT2221 chaperone) in late onset Pompe Disease are anticipated in 1Q21

We continue to believe PROPEL has a high probability of success (see our comprehensive December 2020 slide deck [here](#))

- **This is based on strong pre-clinical rationale, prior phase 1/2 data, and achievable (if not beatable) benchmarks for success in phase 3**
 - Recall, the 6MWD endpoint is the key clinical endpoint; we will be looking for a ~20-30 meter benefit over Lumizyme as a win/upside scenario (with 30+ meters being a homerun; importantly, recall, PROPEL will be enrolling ~70-75% ERT-switch patients / ~30-35% naïve patients)
 - We suspect a significant portion of patients in the Lumizyme control arm could be in the decline phase of the disease (switch patients enrolled in PROPEL were required to be on Lumizyme ≥24 months), and thus our base case assumption is that the Lumizyme arm shows a <10-15m benefit

PROPEL data if positive could help the broader pipeline (including gene therapy optionality) come into greater focus

- **On positive PROPEL results, we see FOLD shares trading to the high-\$20s to mid-\$30s in win/homerun scenarios (versus down to the high-single digits to low-teens in an outright trial failure); there is a mixed scenario that is weighted to the downside**

Where we could be wrong... In our view, the biggest risk for FOLD shares revolves around a clinical setback for PROPEL in the form of a trial failure; we see potential outperformance of the control arm (Lumizyme) as a possible risk for the study

Scenario	AT-GAA Overall Clinical Profile (at 12 months)	JPM Likelihood	JPM Revenue Estimate (Worldwide)	JPM POS	Upside / Downside For FOLD Shares (% move from current levels)	Commentary
Current Model	-		~\$1B+	~80%	-	-
Homerun	Statistically Significant >30 meter benefit on 6MWD relative to Lumizyme (Primary Endpoint) Supportive secondary endpoints No major safety concerns	20%	~1.5B+	~90%	~Mid-30s (Up ~50-60%+)	- Best case scenario that beats expectations - Emerges as a standard of care (best-in-class) Pompe ERT (at a minimum in progressing patients) - Leads to peak revenue and POS increase - Assume peak sales closer to consensus - Potential catalyst for broader pipeline credit - Positive sentiment based momentum on scarcity value
Win / Upside	Statistically Significant ~20-30 meter benefit on 6MWD relative to Lumizyme (Primary Endpoint) Supportive secondary endpoints No major safety concerns	50%	~\$1B+	~90%	~High-\$20s to Low-\$30s (Up ~25-45%+)	- Base case win scenario that leads to upside - Emerges as a standard of care (best-in-class) Pompe ERT (at a minimum in progressing patients) - Leads to POS increase - Potential catalyst for broader pipeline credit - Positive sentiment based momentum on scarcity value
Mixed / Downside	Statistically Significant ~15-20 meter benefit on 6MWD relative to Lumizyme (Primary Endpoint) Mixed / discordant secondary endpoints No major safety concerns	10%	~\$500M+	70-80%	Mid- to High Teens (Down ~10-35%+)	- Positive trial and potential approval still on the table - However market dynamics will be questioned - Likely a downside scenario - Uphill battle for broader pipeline credit - Potential for further sentiment based downside
Trial failure	Misses Statistical Significance (<15-20 meter benefit on 6MWD relative to Lumizyme) Mixed / discordant secondary endpoints Safety signal emerges	20%	-	-	High Single Digits to Low Teens (Down ~40-65%)	- Disaster scenario - Take Pompe program out of the model - Likely trades to the value of the Galafold valuation backstop - Uphill battle for broader pipeline credit - Potential for further sentiment based downside

Other Investment Themes – Anupam Rama

If growth is what you're looking for...

- **Apellis (APLS)**: Coming in 3Q21, we believe phase 3 DERBY & OAKS studies for lead asset pegcetacoplan (APL-2) have a high probability of success in geographic atrophy (GA) and importantly could be a potentially transformational catalyst for the company; indeed, we see a favorable reward risk setup (see our comprehensive slide deck [here](#)).

For investors that are seeking value...

- **Theravance (TBPH)**: Despite several mid- to late-stage readouts expected in the new year (including initial data for COVID-19, ampreloxetine, TD-1473), TBPH shares continue to trade in the middle of the valuation backdrop range of \$15-20/share that is provided by revenues from marketed assets Trelegy and Yupelri; clinical wins are likely to turn sentiment on TBPH shares

If you're looking for a name exposed to a single event...

- **SpringWorks (SWTX)**: While 3-4 niro + BCMA directed therapy trials set to initiate in 2021 (BCMA a key focus in 2020), in our view, SWTX shares in 2021 will be driven by the phase 3 DeFi trial of nirogacestat in desmoid tumors (data anticipated in 2Q-3Q)
- **AlloVir (ALVR)**: In our view, AlloVir's platform and Viralym-M should continue to become increasingly de-risked via POC readouts in prevention and SOT in 2021; we believe AlloVir has the potential to emerge as a core SMID holding over the next several years

Or a launch story...

- **Zai Lab (ZLAB)**: Zai Lab has amassed a significant oncology pipeline via business development and 2 products are currently in a launch phase (Zejula / Optune); in our view, the successful launches for these products should validate regional market dynamics (and have read-through to the broader pipeline)

For investors who are seeking a turnaround story...

- **AnaptysBio (ANAB)**: Following a high-profile setback for former lead asset etokimab in late 2019, ANAB shares had a volatile 1H20. However, recent imsidolimab readouts in GPP have started to shift sentiment, and ANAB shares may be poised for further turnaround if POPLAR data are positive in the new year (expected 1Q21)

Favorite Names: TG Therapeutics – Eric Joseph

TG Therapeutics (TGTX) – Overweight, Dec 2021 PT \$67

We see both UNITY-CLL & NHL programs as near fully de-risked ahead of multiple regulatory updates in 2021, with additional value creation from ublituximab in RMS post the ULTIMATE phase 3 top-line readout

Seeing high probability of approval for U2 (umbralisib + ublituximab) in first-line & r/r CLL and umbralisib in r/r NHL (MZL/FL)

- **Multiple regulatory updates on the near-term horizon**
 - Along with FDA action for umbralisib in r/r MZL and r/r FL (PDUFA dates February 15, 2021, and June 15, 2021), completion of the rolling BLA submission for U2 in both relapsed/refractory and front-line CLL is anticipated in 1H21.
- **Umbralisib commercial oppy of \$450M derisked by updated UNITY-NHL and UNITY-CLL results from ASH**
 - Exhibiting a significant PFS improvement over the control arm (HR:~0.55), U2's efficacy profile was consistent across TN and r/r CLL patients. Further, U2 had a well-tolerated toxicity profile, with discontinuation rates largely in-line with the broader landscape.
 - In the UNITY-NHL study, umbralisib demonstrated an efficacy profile consistent with the broader PI3K class (ORR rates of 49.3%/45.3% in the MZL/FL arms), and a well-differentiated safety profile (minimal colitis, pneumonitis).
- **Compelling optionality from combination work supporting U2 as a backbone therapy in CLL (with Ven/TG-1701)**
 - U2+venetoclax continued to exhibit promising efficacy in CLL (ORR rates maintained at 100% by cycle 7, CR of 42% by cycle 12), and a well-tolerated toxicity profile with Gr3 side-effects largely in line with prior data (no TLS was observed during venetoclax administration).

Value creative potential with ublituximab in RMS in 1H21 following positive phase 3 ULTIMATE top-line readout

- **Forecasting a ~\$2.2B+ commercial oppy for ublituximab with regulatory path de-risked by top-line results from ULTIMATE 1 & 2**
 - Potentially best-in-class anti-CD20 activity, achieving annualized relapse rates (ARR) of <0.10, with relative reductions of 60% and 50% in ULTIMATE 1 & 2, respectively.
 - Ahead of BLA submission targeted for mid-2021, fuller ULTIMATE 1&2 results are anticipated at a medical meeting in 1H21 (we would look to AAN 2021 in April as the earliest potential forum).
- **Ublituximab's administration profile offers potential for meaningful commercial differentiation**
 - Relative to commercially available Ocrevus, ublituximab's shorter infusion time (1hr vs ~2-3hrs) offers a differentiated reimbursement incentive for providers, indicating potential for strong and early adoption.
 - While Kesimpta offers a level of portability as a subcutaneous injection, based on physician feedback, the frequency of doses (once-monthly) and potential hesitancy among patients naïve to self-administration are likely to be hurdles to broad-based uptake.

Where we could be wrong... The biggest risk for TGTX shares are near-term regulatory approvals from umbralisib in NHL and U2 in CLL. Anything short of this would decrease strategic interest in TGTX by the sector over the mid-term to longer term.

Other Investment Themes – Eric Joseph

If growth is what you're looking for...

- **PTC Therapeutics (PTCT):** With the Evrysdi launch underway, looking beyond the near-term update from Study 045 (Translarna for DMD approval in the US), we see PTC518 (Huntington's Disease) as the greater store of value over the course of 2021, with the potential to drive 25-60% upside on initial HV peripheral biomarker results anticipated in 1H21.

For investors who are seeking value...

- **Acceleron (XLRN):** With the Reblozyl launch off to a strong start (246% avg qtr growth through 3Q20), we expect sales to ebb to a slower, but healthy, growth trend in 2021 driven by steady patient adds and expanding average duration on therapy in the US. While 2021 is expected to be more of an execution year for sotatercept in PAH (phase 3 STELLAR study enrollment), with the Reblozyl launch and robust phase 2 PULSAR data providing a solid valuation back stop to shares, we see label expansion initiatives in PAH (early and later invention phase 3 trial HYPERION and ZENITH) and phase 1 anti-fibrosis candidate ACE-1334 increasing as potential stores of value over the next 12-18 months.

If you're looking for a name exposed to a single event...

- **Black Diamond (BDTX):** Initial phase 1 data for lead candidate BDTX-189, including a completed dose-escalation study portion, is anticipated at ASCO 2021. Predominantly comprised of patients with tumors harboring exon20 and allosteric HER2/EGFR mutations, the data should provide a fairly substantive read on anti-tumor activity in at least one or more of the intended phase 2 expansion cohorts, in addition to solidifying PD and safety (where we believe the compound is best-in-class).

Or a launch story...

- **Karyopharm (KPTI):** On the heels of positive results from phase 3 BOSTON study, we see potential label expansion for Xpovio into earlier-line multiple myeloma as fundamentally de-risked ahead of regulatory action anticipated in 1Q21 (sNDA PDUFA of March 19, 2021). Where Xpovio has shown steady commercial performance in both triple refractory multiple myeloma and R/R DLBCL, we expect earlier-line MM launch dynamics unfolding over the course of 2021 to better inform the long-term commercial potential of the Xpovio franchise.

For investors who are seeking a turnaround story...

- **Five Prime (FPRX):** After a surprise success on OS improvement in the phase 2 FIGHT trial (bemarituzumab in gastric/GEJ cancer) and capital raise, a comeback story appears under way for FPRX shares. We believe the data open the opportunity for strategic partnership and potential bema indication expansion in other FGFR2b+ solid tumors. Strategic update and potential data for I/O candidate FPT-155 (expected by YE20) could be additional value drivers for shares.

Q&A

Appendix: Kasimov Comp Sheet

	Company	Ticker	Price	PT	Rating	Mkt Cap	Cash	Debt	EV	Revenues (\$M)			EPS		P/E		P/S	YTD	52 Week		SI
			12/15/2020	YE21		(\$B)	(\$M)	(\$M)	(\$B)	2020E	2021E	2022E	2020E	2021E	2022E	2021E	2021E		High	Low	% of float
Large Caps	AMEX Biotech Index	BTK	\$5,773.92															13.9%	\$6,166	\$3,758	
	NASDAQ Biotech Index	NBI	\$4,833.20															27.6%	\$4,910	\$2,948	
	S&P	SPX	\$3,690.60													26.1x		14.2%	\$3,712	\$2,192	
	Alexion	ALXN	\$157.91	NA	NR	\$34.5	\$2,297	\$2,768	\$35.0	NA	NA	NA	NA	NA	NA	NA	NA	46.0%	\$160.0	\$72.7	2.0%
	Amgen	AMGN	\$230.53	\$222	N	\$134.2	\$12,360	\$34,287	\$156.1	\$25,518	\$26,081	\$27,004	\$16.27	\$17.35	\$19.00	13.3x	5.1x	-4.4%	\$265.0	\$177.1	1.1%
	Biogen	BIIB	\$248.73	\$269	N	\$38.3	\$4,590	\$7,834	\$41.5	\$13,370	\$11,400	\$11,911	\$33.49	\$25.59	\$25.76	9.7x	3.4x	-16.2%	\$375.0	\$223.3	2.7%
	BioMarin	BMRN	\$85.03	\$131	OW	\$15.4	\$1,506	\$1,502	\$15.4	\$1,861	\$2,004	\$2,419	\$1.64	\$1.93	\$3.15	44.0x	7.7x	0.6%	\$131.9	\$68.3	6.6%
	BioNTech	BNTX	\$111.20	\$90	N	\$26.8	\$990	\$0	\$21.0	\$153	\$973	\$1,923	(\$2.62)	\$1.05	\$4.21	106.0x	27.5x	229.3%	\$131.0	\$27.6	NA
	Gilead	GILD	\$59.43	\$74	N	\$74.5	\$26,049	\$29,290	\$77.8	\$23,786	\$23,121	\$21,748	\$6.58	\$6.89	\$6.49	8.6x	3.2x	-8.5%	\$86.0	\$57.0	1.7%
	Incyte	INCY	\$86.27	\$98	N	\$18.9	\$1,735	\$71	\$17.2	\$2,532	\$2,809	\$3,359	(\$0.58)	\$3.84	\$5.37	22.4x	6.7x	-1.2%	\$110.4	\$62.5	2.5%
	Moderna	MRNA	\$147.22	\$89	N	\$58.3	\$3,276	\$212	\$55.2	\$357	\$3,573	\$4,406	(\$2.13)	\$3.60	\$4.90	40.9x	16.3x	652.7%	\$178.5	\$17.7	8.5%
	Novocure	NVCR	\$157.10	\$88	N	\$16.0	\$234	\$13	\$15.8	\$489	\$560	\$635	\$0.27	\$0.35	\$0.32	443.9x	28.6x	86.4%	\$174.6	\$53.4	5.9%
	Regeneron	REGN	\$491.79	\$550	N	\$52.5	\$5,901	\$2,695	\$49.3	\$8,825	\$10,332	\$12,605	\$32.14	\$39.18	\$52.53	12.6x	5.1x	31.0%	\$664.6	\$328.1	2.1%
	Seagen	SGEN	\$196.02	\$182	OW	\$35.3	\$1,718	\$77	\$33.7	\$2,144	\$1,707	\$2,532	\$3.34	\$0.23	\$2.70	864.6x	20.7x	71.6%	\$213.9	\$90.6	3.2%
	Vertex	VRTX	\$228.81	\$279	OW	\$59.5	\$6,151	\$547	\$53.9	\$6,134	\$6,683	\$7,453	\$10.46	\$11.32	\$13.02	20.2x	8.9x	4.5%	\$306.1	\$197.5	1.0%
Large-Cap Average:						\$47	\$5,567	\$6,608	\$47.7	\$7,743	\$8,113	\$8,727	\$8.99	\$10.12	\$12.50	12.9x**	5.1x**	91.0%			3.4%
															**Average P/E & P/S for AMGN, BIIB, GILD, REGN & VRTX						
SMid Caps	Acadia	ACAD	\$52.57	\$60	OW	\$8.4	\$644	\$49	\$7.8	\$448	\$768	\$1,076	(\$1.81)	(\$0.29)	\$1.02	NM	10.9x	22.9%	\$58.7	\$30.0	6.7%
	Alkermes	ALKS	\$22.10	\$21	N	\$3.5	\$569	\$386	\$3.3	\$1,012	\$1,128	\$1,296	\$0.37	\$0.53	\$1.36	41.7x	3.1x	8.3%	\$22.2	\$12.0	7.3%
	Allogene	ALLO	\$28.23	\$37	N	\$4.0	\$841	\$54	\$3.2	\$0	\$0	\$0	(\$2.20)	(\$2.35)	(\$2.59)	NM	NM	8.7%	\$55.0	\$17.4	16.6%
	bluebird bio	BLUE	\$44.36	\$76	OW	\$2.9	\$1,438	\$195	\$1.7	\$261	\$372	\$526	(\$10.08)	(\$8.91)	(\$8.11)	NM	7.9x	-49.4%	\$99.4	\$39.0	10.4%
	Clovis Oncology	CLVS	\$5.11	NA	N	\$0.5	\$225	\$543	\$0.8	\$161	\$210	\$328	(\$4.24)	(\$3.09)	(\$1.63)	NM	2.1x	-51.0%	\$13.3	\$3.6	39.2%
	Editas	EDIT	\$64.93	\$30	N	\$4.0	\$541	\$27	\$3.5	\$88	\$10	\$10	(\$1.89)	(\$3.89)	(\$3.81)	NM	404.7x	119.3%	\$67.7	\$14.0	18.0%
	Global Blood Therapeutics	GBT	\$44.86	\$70	OW	\$2.8	\$535	\$159	\$2.4	\$125	\$218	\$433	(\$3.91)	(\$2.95)	(\$0.23)	NM	12.7x	-43.6%	\$87.5	\$36.5	19.3%
	GW Pharmaceuticals	GWPH	\$116.95	\$190	OW	\$3.6	\$480	\$31	\$3.2	\$524	\$753	\$972	(\$0.16)	\$0.15	\$0.46	789.3x	4.8x	11.8%	\$144.0	\$68.0	NA
	Jounce Therapeutics	JNCE	\$6.84	NA	UW	\$0.3	\$104	\$18	\$0.2	\$35	\$85	\$0	(\$1.72)	(\$0.30)	(\$1.91)	NM	3.2x	-21.6%	\$11.7	\$2.9	7.7%
	Legend Biotech	LEGN	\$30.10	\$50	OW	\$4.0	\$575	\$0	\$3.4	\$47	\$62	\$225	(\$1.38)	(\$1.15)	(\$0.83)	NM	64.4x	-18.6%*	\$43.2	\$24.6	NA
	Puma Biotechnology	PBYI	\$10.74	NA	UW	\$0.4	\$109	\$121	\$0.4	\$225	\$239	\$262	(\$0.94)	(\$0.30)	\$0.41	NM	1.8x	22.7%	\$15.0	\$5.5	22.7%
	Sage Therapeutics	SAGE	\$71.87	\$101	OW	\$3.7	\$669	\$30	\$3.1	\$7	\$16	\$58	(\$9.54)	(\$9.17)	(\$9.89)	NM	231.7x	-0.4%	\$86.7	\$25.0	10.6%
	Ultragenyx	RARE	\$158.88	\$101	OW	\$10.5	\$766	\$44	\$9.8	\$240	\$322	\$433	(\$3.94)	(\$4.23)	(\$5.15)	NM	32.6x	272.0%	\$159.3	\$32.0	10.9%
SMid-Cap Average:						\$4	\$577	\$127	\$3	\$244	\$322	\$432	(\$3.19)	(\$2.77)	(\$2.38)	415.5x	65.0x	21.6%			15.4%

*LEGN's YTD performance is based on 06/05/2020

Appendix: Fye Comp Sheet

Company Name	Ticker	Rating	Price	Price Target	Market Cap (MM)	Enterprise Value	EV/Sales	Net Debt (MM)	YTD	52 Week High	52 Week Low	Short Interest % of Float
Amarin	AMRN	N	\$5.27	n/a	2,048	1,512	2.6x	(536)	-75.4%	26.12	3.36	-
Aprea Therapeutics	APRE	N	\$29.73	31.00	630	529	N/A	(101)	-35.2%	53.11	19.67	6%
Aptinyx	APTX	N	\$3.56	n/a	225	121	N/A	(104)	4.1%	6.47	1.60	3%
Arena Pharmaceuticals, Inc.	ARNA	OW	\$71.26	83.00	4,145	3,024	N/A	(1,121)	56.9%	90.19	32.95	6%
Ascendis Pharma A/S	ASND	OW	\$175.79	177.00	9,390	8,632	N/A	(758)	26.4%	183.98	92.00	-
Axcella Health	AXLA	N	\$5.92	8.00	222	130	N/A	(92)	47.6%	7.73	2.25	2%
BioCryst Pharmaceuticals	BCRX	OW	\$8.31	8.00	1,467	1,405	N/A	(63)	140.9%	8.99	1.58	21%
Crinetics	CRNX	N	\$13.95	23.00	459	277	N/A	(182)	-44.4%	26.67	10.63	6%
Deciphera	DCPH	N	\$56.40	65.00	3,211	2,658	117.6x	(553)	-9.4%	71.11	33.10	9%
Denali	DNLI	OW	\$81.89	65.00	9,820	8,908	N/A	(912)	370.1%	85.92	12.39	10%
Emergent BioSolutions	EBS	N	\$89.29	102.00	4,732	5,222	3.9x	490	65.5%	137.61	46.37	4%
Esperion Therapeutics	ESPR	UW	\$28.17	32.00	785	578	2.6x	(207)	-52.8%	76.98	23.90	35%
Halozyme Therapeutics	HALO	OW	\$42.20	33.00	5,705	5,752	28.8x	47	138.0%	44.53	12.71	12%
ImmunoGen	IMGN	N	\$7.44	8.00	1,395	1,232	13.5x	(164)	45.7%	7.68	1.95	11%
Intra-Cellular Therapies	ITCI	OW	\$26.50	36.00	2,124	1,431	137.4x	(693)	-22.8%	43.56	10.94	9%
Ionis Pharmaceuticals	IONS	N	\$51.49	62.00	7,199	5,913	6.3x	(1,285)	-14.8%	66.22	39.32	5%
Jazz Pharmaceuticals	JAZZ	OW	\$152.58	183.00	8,501	8,827	3.9x	326	2.2%	158.98	86.88	7%
Kaleido Pharmaceuticals	KLDO	UW	\$9.94	6.00	358	324	N/A	(34)	98.0%	11.89	2.82	25%
Lexicon Pharmaceuticals	LXRX	UW	\$3.07	n/a	434	343	N/A	(91)	-26.0%	5.03	1.03	13%
Magenta	MGTA	OW	\$7.28	13.00	352	190	N/A	(162)	-52.0%	16.19	5.76	7%
Mersana	MRSN	N	\$23.56	20.00	1,614	1,360	N/A	(254)	311.2%	26.71	3.72	11%
Nektar Therapeutics	NKTR	N	\$18.43	26.00	3,306	2,505	15.3x	(802)	-14.6%	28.60	13.63	14%
Radius Health	RDUS	N	\$15.46	23.00	720	809	3.5x	89	-23.3%	23.20	10.15	16%
Rubius Therapeutics	RUBY	N	\$7.78	6.00	630	540	N/A	(90)	-18.1%	14.44	3.35	25%
Stoke Therapeutics	STOK	N	\$55.85	42.00	2,024	1,833	N/A	(191)	97.2%	58.89	15.82	16%
Tricida	TCDA	UW	\$7.77	6.00	390	280	N/A	(110)	-79.4%	40.29	3.74	16%
United Therapeutics	UTHR	OW	\$148.21	148.00	6,587	4,579	3.2x	(2,008)	68.3%	148.47	75.58	5%
Zymergen	ZYME	N	\$51.51	49.00	2,361	1,930	N/A	(431)	13.3%	56.73	20.33	3%

Source: Bloomberg Finance L.P., J.P. Morgan estimates, priced as of 12/15/2020

Appendix: Rama Comp Sheet

Company Name	Ticker	Rating	Price	Price Target	Market Cap (MM)	Cash (MM)	YTD	52 Week High	52 Week Low	Short Interest % of Float
Aeglea Biotherapeutics	AGLE	OW	\$9.09	\$11.00	436	140	19.0%	11.38	3.50	6%
Agios Pharmaceuticals	AGIO	OW	\$34.39	\$64.00	2,382	722	-28.0%	56.75	27.77	8%
Alnylam Pharmaceuticals	ALNY	N	\$135.70	\$145.00	15,766	1,834	17.8%	167.33	84.97	3%
Allovir	ALVR	OW	\$41.91	\$50.00	2,729	378	NA	47.55	18.15	NA
Amicus Therapeutics	FOLD	OW	\$23.86	\$29.00	6,218	509	145.0%	24.62	6.25	11%
AnaptysBio	ANAB	UW	\$24.48	\$19.00	669	338	50.6%	31.29	12.06	14%
Annexon Biosciences	ANNX	OW	\$25.30	\$32.00	965	371	NA	31.84	15.33	4%
Apellis Pharmaceuticals	APLS	OW	\$52.77	\$87.00	3,997	416	72.3%	56.32	16.85	15%
Atara Biotherapeutics	ATRA	N	\$23.12	\$22.00	1,903	327	40.4%	28.20	4.52	16%
BridgeBio Pharma	BBIO	NR	\$60.99	NR	7,484	711	74.0%	64.32	14.23	15%
ChemoCentryx Inc.	CCXI	N	\$65.16	\$60.00	4,510	471	64.8%	65.43	30.72	10%
Constellation	CNST	OW	\$32.62	\$46.00	1,552	489	-30.8%	50.90	17.00	21%
Dyne Therapeutics	DYN	OW	\$20.45	\$27.00	929	380	NA	27.11	15.60	10%
Eidos Therapeutics	EIDX	NR	\$112.28	NR	4,363	147	95.6%	119.41	28.39	5%
Frequency Therapeutics	FREQ	N	\$37.99	\$27.00	1,282	224	116.7%	39.60	14.50	14%
Generation Bio	GBIO	OW	\$34.67	\$34.00	1,613	280	NA	55.72	17.00	9%
G1 Therapeutics	GTHX	OW	\$19.30	\$21.00	734	238	-27.0%	31.38	8.80	26%
Ideaya Biosciences	IDYA	OW	\$14.52	\$28.00	422	279	93.6%	19.97	2.95	4%
**Idera Pharmaceuticals	IDRA	OW	\$4.25	\$9.00	150	29	133.5%	5.37	0.81	2%
Infinity	INFI	UW	\$2.09	-	134	41	117.7%	3.13	0.60	0%
iTeos Therapeutics	ITOS	OW	\$31.23	\$40.00	1,094	340	NA	34.33	17.50	3%
InflaRx	IFRX	UW	\$4.83	-	136	45	22.0%	9.70	2.52	2%
Kiniksa	KNSA	OW	\$19.58	\$35.00	1,333	364	77.0%	28.67	10.30	9%
Kodiak Sciences	KOD	N	\$133.06	\$125.00	6,750	348	84.9%	141.98	35.49	12%
Mirati Therapeutics	MRTX	OW	\$233.29	\$238.00	11,876	415	81.0%	249.42	66.01	9%
Neurocrine Biosciences	NBIX	N	\$95.32	\$121.00	8,906	945	-11.3%	136.27	72.14	5%
Olema Oncology	OLMA	OW	\$39.26	\$52.00	1,577	368	NA	60.27	38.60	NA
Orchard Therapeutics	ORTX	OW	\$4.49	\$14.00	439	201	-67.3%	15.93	3.76	NA
ORIC Pharmaceuticals	ORIC	OW	\$34.84	\$38.00	1,266	187	NA	40.67	16.00	9%
Oyster Point Pharma	OYST	OW	\$19.96	\$47.00	516	214	-18.3%	41.37	16.00	6%
Passage Bio	PASG	N	\$26.86	\$25.00	1,223	336	NA	38.23	8.09	7%
Replimune	REPL	OW	\$42.39	\$56.00	1,948	245	195.4%	54.85	8.58	4%
***Rigel Pharmaceuticals	RIGL	N	\$3.23	-	546	73	50.9%	5.24	1.23	14%
Sarepta Therapeutics	SRPT	OW	\$167.95	\$185.00	13,253	1,826	30.2%	178.66	78.06	12%
Solid Biosciences	SLDB	UW	\$5.91	-	357	25	32.8%	6.85	1.93	18%
Theravance	TBPH	OW	\$19.74	\$28.00	NA	358	-23.8%	31.54	14.48	NA
SpringWorks	SWTX	OW	\$75.85	\$57.00	3,699	277	97.1%	76.85	19.50	12%
Vir Biotechnology	VIR	N	\$33.01	\$28.00	4,205	827	162.5%	75.00	12.00	18%
Y-mAbs Therapeutics	YMAB	OW	\$53.88	\$57.00	2,183	131	72.4%	55.22	14.16	11%
Zai Lab	ZLAB	OW	\$112.92	\$111.00	9,940	464	171.5%	114.38	38.06	NA

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**Company covered by Matt Bannon (matthew.bannon@jpmorgan.com | 212-622-0001)

Appendix: Joseph Comp Sheet

Company Name	Ticker	Rating	Price Dec. 15	Price Target	Market Cap (MM)	Cash (MM)	Net Debt (MM)	52 Week		Short Interest % of Float
Acceleron	XLRN	OW	\$128.87	133.00	7,765	882	22	132.74	50.04	6%
Akebia	AKBA	N	\$3.08	-	445	269	105	13.71	2.09	17%
Akero	AKRO	OW	\$29.11	45.00	1,011	292	2	41.16	10.78	10%
Aligos	ALGS	OW	\$22.89	33.00	873	70	13	28.69	12.82	10%
Atea	AVIR	OW	\$30.71	45.00	2,537	105	0	36.65	24.15	16%
Autolus	AUTL	OW	\$8.13	24.00	425	178	53	17.19	3.00	N/A
Beam Therapeutics	BEAM	OW	\$77.70	38.00	4,504	202	34	86.45	13.00	11%
Black Diamond	BDTX	OW	\$34.41	43.00	1,239	269	0	46.25	17.63	14%
Enanta Pharmaceuticals	ENTA	UW	\$42.82	44.00	860	419	8	67.88	38.40	13%
Five Prime Therapeutics	FPRX	N	\$19.44	14.00	853	111	46	24.70	1.75	2%
Freeline Therapeutics	FRLN	OW	\$17.40	27.00	624	73	-	21.69	14.62	N/A
Ironwood Pharmaceuticals	IRWD	N	\$11.97	11.00	1,916	308	448	14.10	7.99	13%
Karyopharm Therapeutics	KPTI	OW	\$14.95	37.00	1,100	263	129	29.61	13.39	23%
Myovant	MYOV	OW	\$24.20	27.00	2,192	111	265	28.00	5.98	8%
Novavax	NVAX	OW	\$126.22	215.00	8,035	504	441	189.40	3.65	14%
Nurix Therapeutics	NRIX	OW	\$43.67	34.00	1,697	354	0	52.38	15.21	7%
ObsEva	OBSV	UW	\$2.21	-	122	51	27	6.30	1.63	3%
Precigen Inc	PGEN	N	\$7.17	6.00	1,330	75	248	9.50	1.26	37%
Precision Biosciences	DTIL	N	\$8.01	10.00	420	181	0	16.58	4.46	5%
PTC Therapeutics	PTCT	OW	\$66.37	77.00	4,529	1,141	414	68.81	30.79	7%
Relay Therapeutics	RLAY	N	\$45.72	36.00	4,114	713	24	57.59	20.00	26%
Revolution	RVMD	N	\$45.44	33.00	3,016	466	33	47.14	17.35	6%
Rocket	RCKT	OW	\$58.55	43.00	3,545	229	69	63.99	9.01	23%
Sangamo Therapeutics	SGMO	N	\$12.98	13.00	1,836	695	42	15.69	4.81	17%
TG Therapeutics	TGTX	OW	\$51.17	67.00	7,052	254	41	51.50	6.34	11%
Urogen	URGN	N	\$18.44	26.00	407	126	3	35.21	13.12	16%
Urovant	UROV	N	\$16.10	16.00	511	74	179	\$16.25	6.55	3%
Ziopharm Oncology	ZIOP	UW	\$2.83	-	606	135	3	5.27	1.80	16%

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