February 28, 2018

OUTPERFORM

Reason for report:

EARNINGS

Joseph P. Schwartz
(617) 918-4575
Joseph.Schwartz@Leerink.com

Dae Gon Ha, Ph.D.
(617) 918-4093
Daegon.Ha@Leerink.com

LEERINK

AMICUS THERAPEUTICS, INC.

4Q Recap: All Eyes Set on Pompe/Fabry Updates in 2Q/3Q; PT to $22

• **Bottom Line:** 2017 caps Galafold's (migalastat) first yr. of commercialization in the EU paving the way for additional growth WW (with regulatory decisions anticipated from Japan and the US) complemented by updates from the company's other drug candidate - ATB200/AT2221 (Pompe disease). Regarding the latter, Ph.1/2 study will enroll 4-6 more pts. while observational studies like POM-002 (retrospective) and POM-003 (STRIDE; prospective) can contextualize both natural history and comparator data from pts. on the current standard of care (SoC). Regulatory update in 2Q18 could determine whether an early filing (therefore earlier mkt. entry) is a possibility in Pompe. Our updated model incorporates 4Q financial results. Reiterate OP on FOLD but decreasing our PT to $22 (from $24 previously) on increased expense assumptions.

• **Galafold's growth in 2017 carries over into 2018; FY2018 guidance of $75M-$85M does not include US or Japan contributions.** In 2017, Amicus reached its goal of treating 300 commercial pts. Within the first 2 months of 2018, Amicus has already added 50 new pts. across the approved territories. Galafold has started 2018 with a bang and we anticipate sales to continue its upward trajectory towards mgmt. guidance of $75M-$85M. To this point, with 310+ pts. treated as of YE17, current guidance does not appear to reflect much growth (assuming annual Galafold price of $200K-$300K) nor does the guidance include contributions from Japan (approval in 1H18) and the US (approval in 3Q18). However, we believe mgmt. is taking a more conservative approach in its initial guidance, and we expect further refinement as they gain further clarity on commercial sales in both already-approved and soon-to-be approved territories.

• **Pompe update in 2Q could open doors to an earlier mkt. entry.** The company's enzyme-chaperone candidate - ATB200/AT2221 - has generated promising clinical data thus far in the Ph.1/2 study (LINK). Mgmt. today announced the study will enroll 4-6 additional pts. to gain further insight on the drug's clinical profile. Meanwhile, observational studies - POM-002 (n~200) and POM-003 (n~100; potential run-in for upcoming Ph.3 study) - could provide important nat. history and comparator data to the ongoing Ph.1/2 study. As previously guided, Amicus will meet with regulators with an update in 2Q. While it is early to speculate, mgmt. believes a range of outcomes are possible from the meeting but the company remains laser-focused on getting the fastest approval pathway for Pompe pts. Our model already reflects an early launch possibility ascribing 50% probability of success to the 2019E mkt. entry (vs. 75% PoS for 2021E launch).

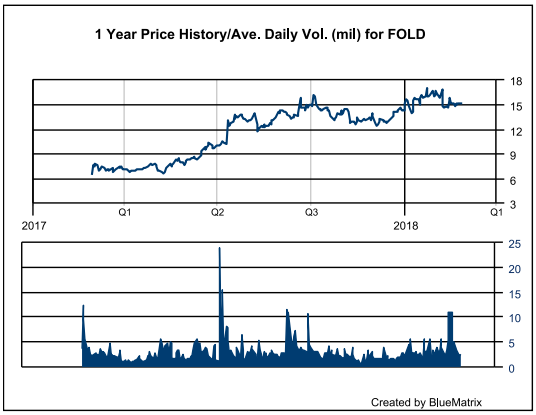
Key Stats:	**(NASDAQ: FOLD)**
Sector:	**Biotechnology**
S&P 500 Health Care Index:	**988.06**
Price :	**$14.29**
Price Target:	$22.00 from $24.00
Methodology:	DCF, 10% discount rate, 2% terminal growth
52 Week High:	$17.40
52 Week Low:	$5.90
Shares Outstanding (mil):	166.9
Market Capitalization (mil):	2,385.0
Book Value/Share:	$0.02
Cash Per Share:	$0.65
Net Debt to Total Capital:	17%
Convertibles:	Yes
Dividend (ann):	$0.00
Dividend Yield:	0.0%

Completion: February 28, 2018, 12:41PM EDT.
Distribution: February 28, 2018, 12:41PM EDT.
Cash Per Share: Net cash
General: intra day price

Dec Yr	1Q	2Q	3Q	4Q	FY Rev	1Q	2Q	3Q	4Q	FY EPS	P/E
2017A	**$4.2**	**$7.2**	**$10.9**	**$14.7**	**$36.9**	**($0.39)**	**($0.34)**	**($0.69)**	**($0.41)**	**($1.85)**	**NM**
2018E - New	**$16.3**	**$19.0**	**$22.7**	**$26.4**	**$84.4**	**($0.37)**	**($0.37)**	**($0.37)**	**($0.36)**	**($1.47)**	**NM**
2018E - Old	$16.3	$18.1	$21.0	$23.7	$79.1	($0.32)	($0.32)	($0.32)	($0.32)	($1.29)	NM
2019E - New	**--**	**--**	**--**	**--**	**$168.3**	**--**	**--**	**--**	**--**	**($1.06)**	**NM**
2019E - Old	--	--	--	--	$168.3	--	--	--	--	($0.93)	NM

Source: Company Information and Leerink Partners LLC Research
Revenues in MM; GAAP EPS.

• **The fate of ATB101/migalastat (Fabry disease) hinges on gene therapy?** Combining the past experiences with migalastat (Fabry disease) and ATB200/AT2221, Amicus has been developing ATB101/ migalastat to address the entire Fabry mkt. that migalastat alone cannot address. While development remains ongoing with today's press release highlighting a potential entry into the clinic in 2019, mgmt. cited ongoing due diligence of new technologies as a gating factor for this program. CEO John Crowley has both today and previously (on CNBC; LINK) mentioned his interest in gene therapy as it can potentially deliver a cure for rare disease pts. To that end, we note that the cash runway guidance of "into at least 2021" dose not include BD activities.



• **Updating our model to incorporate 4Q revenues and EPS of $14.7M/(($0.41), respectively.** 4Q revenues beat our/Street expectations by 4-8% while EPS was lower than our/Street expectations by 24-37%. R&D and SG&A expenses rose 13% and 32% QoQ, respectively, presumably as the company rolls out POM-002 and POM-003 studies and prepare for commercialization in the US and Japan. Our 2018 OpEx factors further increase in expense although at a more modest rate of 2-5% QoQ. We have also adjusted our migalastat revenues in-line with $75M-$85M guidance range that does not factor-in Japan or the US.

INVESTMENT THESIS

Amicus Therapeutics Inc. (FOLD) is a biopharmaceutical company focused on the discovery and development of pharmacological chaperones and next-generation enzyme replacement therapies (ERTs) leveraging its Chaperone Advanced Replacement Technology (CHART). FOLD's lead product candidate Galafold (migalastat HCl) is approved and continues its commercial launch ex-US. Recall, FOLD reported disappointing 6-month Phase III results from its first U.S. registration trial (Study '011) in December 2012, but 18-24 month data showed that patients experienced improvements in kidney, cardiac, and gastrointestinal function. FOLD reported positive results from its European registration trial (Study '012) wherein a little over half of patients were switched to Galafold from Fabrazyme or Replagal ERTs for 18 months. Galafold received EMA approval in 2Q16 and has commenced commercial activities there. Elsewhere, Amicus has submitted a Japanese NDA with regulatory decision anticipated in 1H18; US NDA was also submitted YE17 with a decision anticipated in 3Q18 (PDUFA date: Aug 13). Longer term, we see more potential value than is currently reflected in the stock for FOLD's pharmacological chaperones co-formulated with its proprietary next-generation enzyme replacement therapies (ERT). FOLD generated positive Phase I/II data out to 12 months using its next-generation ERT and chaperone combo for Pompe disease showing either disease stabilization or improvements across treated cohorts irrespective of prior treatment experience and ambulatory status. We believe FOLD is in the process of transitioning from just a chaperone monotherapy company (i.e., Migalastat/Galafold) to a higher value chaperone-ERT co-formulation company with prospects similar to companies such as BMRN (OP). FOLD ultimately aims to offer an enzyme replacement therapy (ERT) that is optimized with a co-formulated pharmacologic chaperone for every lysosomal storage disorder (LSD) but also remains open to other modalities such as gene therapy.

VALUATION

Our price target in 12 months is $22/share and is based on a discounted cash flow (DCF) analysis of probability-weighted sales using a 10% discount rate, 2% terminal growth rate, plus net cash.

RISKS TO VALUATION

Risks include the potential for disappointing clinical data, regulatory setbacks, commercial shortfalls, and dilutive financing. Since FOLD presently has two late-stage product candidates, any of those possible setbacks may impact the stock significantly.

FOLD 4Q17 Results ('000 except EPS)	Consensus 4Q17E	Leerink 4Q17E	4Q17A	Consensus Variance	Leerink Variance
Revenues	**13,600.0**	**14,145.6**	**14,729.0**	*1,129.0*	*583.4*
				-	-
COGS	2,100.0	2,121.8	2,610.0	*510.0*	*488.2*
R&D	39,900.0	38,609.0	45,808.0	*5,908.0*	*7,199.1*
G&A	23,000.0	23,811.7	28,581.0	*5,581.0*	*4,769.3*
				-	-
Operating Income	**(49,100.0)**	**(51,084.7)**	**(67,677.0)**	*(18,577.0)*	*(16,592.3)*
				-	-
Interest Expense	(3,400.0)	(3,250.0)	(3,026.0)	*374.0*	*224.0*
Tax Expense	-	-	(541.0)	*(541.0)*	*(541.0)*
				-	-
Net Income	**(51,400.0)**	**(54,334.7)**	**(69,208.0)**	*(17,808.0)*	*(14,873.3)*
				-	-
				-	-
GAAP EPS	**$ (0.30)**	**$ (0.33)**	**$ (0.41)**	*(0.1)*	*(0.08)*
Shares Oustanding 4Q17		**164,000**	**166,852**		

Source: FactSet, Company reports and Leerink Partners estimates

FOLD P&L ($K)	2010A	2011A	2012A	2013A	2014A	2015A	2016A	1Q17A	2Q17A	3Q17A	4Q17A	2017A	1Q18E	2Q18E	3Q18E	4Q18E	2018E	2019E	2020E
Revenues																			
Reseach	-	14,794	11,591	363	1,224	-	-					-					-	-	-
Collaborations	922	6,640	6,820	-	-	-	-					-					-	-	-
Galafold Sales	-	-	-	-	-	-	4,958	4,169	7,158	10,874	14,729	36,930	16,308	19,038	22,661	26,365	84,372	155,432	201,152
Pipeline ERT Sales	-	-	-	-	-	-	-					-					-	12,822	27,301
Total revenue	**922**	**21,434**	**18,411**	**363**	**1,224**	**-**	**4,958**	**4,169**	**7,158**	**10,874**	**14,729**	**36,930**	**16,308**	**19,038**	**22,661**	**26,365**	**84,372**	**168,253**	**228,453**
Operating expenses																			
COGS	-	-	-	-	-	-	833	775	1,061	1,790	2,610	6,236	2,756	3,217	3,829	4,455	14,257	28,432	34,268
R&D	39,042	50,855	50,273	41,944	47,624	76,943	104,793	30,876	31,985	40,641	45,808	149,310	47,640	48,831	50,296	51,302	198,070	200,221	210,177
G&A	15,660	19,880	19,364	18,893	20,717	47,269	71,151	19,132	19,311	21,647	28,581	88,671	30,010	31,511	32,771	33,754	128,046	131,237	134,788
D&A	2,058	1,586	1,705	1,719	1,546	1,833	3,242	823	812	851	1,107	3,593	750	750	750	750	3,000	3,000	3,000
Restructuring charges/CC	-	-	-	1,988	37	4,392	6,829	4,578	1,050	221,177	4,300	231,105					-	-	-
Total operating expenses	**56,760**	**72,321**	**71,342**	**64,544**	**69,924**	**130,437**	**186,015**	**55,409**	**53,158**	**284,316**	**79,796**	**472,679**	**78,400**	**81,092**	**83,817**	**85,806**	**329,116**	**334,459**	**347,965**
Operating income (loss)	(55,838)	(50,887)	(52,931)	(64,181)	(68,700)	(130,437)	(181,890)	(52,015)	(47,061)	(275,232)	(67,677)	(441,985)	(64,848)	(65,271)	(64,985)	(63,897)	(259,002)	(194,638)	(153,779)
Other																			
Interest income	156	160	316	174	223	929	1,602	759	753	1,190	1,394	4,096	750	750	750	750	3,000	2,500	2,000
Interest expenses	(260)	(148)	(89)	(46)	(1,484)	(1,578)	(5,398)	(4,290)	(4,179)	(4,351)	(4,420)	(17,240)	(4,000)	(4,000)	(4,000)	(4,000)	(16,000)	(16,000)	(16,000)
Change in fair value of warrant liability	(1,410)	2,764	653	908	-	-	-					-	-	-	-	-	-	-	-
Other	1,277	70	21	-	(77)	(1,032)	(18,095)	610	2,400	2,044	954	6,008	-	-	-	-	-	-	-
Total other income (expense)	(237)	2,846	901	1,036	(1,338)	(1,681)	(21,891)	(2,921)	(1,026)	(1,117)	(2,072)	(7,136)	(3,250)	(3,250)	(3,250)	(3,250)	(13,000)	(13,500)	(14,000)
EBIT	(56,075)	(48,041)	(52,030)	(63,145)	(70,038)	(132,118)	(203,781)	(54,936)	(48,087)	(276,349)	(69,749)	(449,121)	(68,098)	(68,521)	(68,235)	(67,147)	(272,002)	(208,138)	(167,779)
Income tax	(1,139)	(3,629)	(3,245)	(3,512)	(1,113)	-	(3,739)	56	49	(164,683)	(541)	(165,119)	-	-	-	-	-	-	-
Net Income (loss)	**(54,936)**	**(44,412)**	**(48,785)**	**(59,633)**	**(68,925)**	**(132,118)**	**(200,042)**	**(54,992)**	**(48,136)**	**(111,666)**	**(69,208)**	**(284,002)**	**(68,098)**	**(68,521)**	**(68,235)**	**(67,147)**	**(272,002)**	**(208,138)**	**(167,779)**
EPS (Basic)	**(1.98)**	**(1.28)**	**(1.07)**	**(1.16)**	**(0.93)**	**(1.20)**	**(1.49)**	**(0.39)**	**(0.34)**	**(0.69)**	**(0.41)**	**(1.85)**	**(0.37)**	**(0.37)**	**(0.37)**	**(0.36)**	**(1.47)**	**(1.06)**	**(0.85)**
EPS (Diluted)	**(1.98)**	**(1.28)**	**(1.07)**	**(1.16)**	**(0.93)**	**(1.20)**	**(1.49)**	**(0.39)**	**(0.34)**	**(0.69)**	**(0.41)**	**(1.85)**	**(0.37)**	**(0.37)**	**(0.37)**	**(0.36)**	**(1.47)**	**(1.06)**	**(0.85)**
Common shares outstanding (Basic)	27,735	34,570	45,565	51,286	74,352	109,924	134,412	142,771	143,001	160,797	166,852	153,355	184,087	185,087	186,087	187,087	185,587	196,450	196,750
Common shares outstanding (Diluted)	27,735	34,570	45,565	51,286	74,352	109,924	134,412	142,771	143,001	160,797	166,852	153,355	184,087	185,087	186,087	187,087	185,587	196,450	196,750

Balance Sheet	2010E	2011A	2012A	2013A	2014A	2015A	2016A	1Q17A	2Q17A	3Q17A	4Q17A	2017A	1Q18E	2Q18E	3Q18E	4Q18E	2018E	2019E	2020E
Cash and equivalents	**107,400**	**56,000**	**99,122**	**81,948**	**169,167**	**213,826**	**330,351**	**279,842**	**227,232**	**426,630**	**358,562**	**358,562**	**568,545**	**508,386**	**448,796**	**390,502**	**390,502**	**416,861**	**284,985**
Convertible debt	-	-	-	-	14,473	-	250,000	250,000	250,000	250,000	250,000	250,000	250,000	250,000	250,000	250,000	250,000	250,000	250,000
Credit facility						50,000	-	-	-	-	-	-	-	-	-	-	-	-	-
Net cash	107,400	56,000	99,122	81,948	183,640	163,826	80,351	29,842	(22,768)	176,630	108,562	108,562	318,545	258,386	198,796	140,502	140,502	166,861	34,985
Net income	(54,936)	(44,412)	(48,785)	(59,633)	(68,925)	(132,118)	(200,042)	(54,992)	(48,136)	(111,666)	(69,208)	(284,002)	(68,098)	(68,521)	(68,235)	(67,147)	(272,002)	(208,138)	(167,779)
Stock-based compensation	6,737	8,488	8,467	6,764	8,314	14,905	17,504	6,030	5,537	5,500	7,401	24,468	8,082	8,362	8,645	8,852	33,941	34,497	35,903
Other	77,375	(15,476)	83,440	35,696	147,829	161,872	300,210	(143,233)	84,549	132,488	-	73,804	270,000				270,000	200,000	-
Change in cash	**29,176**	**(51,400)**	**43,122**	**(17,174)**	**87,218**	**44,659**	**117,672**	**(192,195)**	**41,950**	**26,322**	**(61,807)**	**(185,730)**	**209,983**	**(60,159)**	**(59,590)**	**(58,294)**	**31,940**	**26,360**	**(131,876)**
Interest rate	0.2%	0.2%	0.4%	0.2%	0.2%	0.5%	0.2%	0.2%	0.3%	0.4%	0.4%	0.3%	0.2%	0.1%	0.2%	0.2%	0.2%	0.2%	0.2%
Interest rate (debt)																			

Source: Company Reports, Leerink Partners Research

FOLD DCF Analysis	2014	2015	2016	2017E	2018E	2019E	2020E	2021E	2022E	2023E	2024E	2025E	2026E	2027E	2028E	2029E	2030E	TV
Net income ($MM)	(68.9)	(132.1)	(200.0)	(284.0)	(272.0)	(208.1)	(167.8)	(42.2)	85.0	227.5	366.1	465.3	488.1	473.2	464.9	498.3	535.3	
Non cash charges	8.3	14.9	17.5	24.5	33.9	34.5	35.9	37.2	38.8	40.2	40.1	38.7	37.7	36.6	35.9	35.8	35.7	
FCF ($MM)	(60.6)	(117.2)	(182.5)	(259.5)	(238.1)	(173.6)	(131.9)	(5.0)	123.8	267.7	406.2	503.9	525.8	509.7	500.9	534.1	571.0	7,280.3
Discount Periods	-	-	-	-	-	1.0	2.0	3.0	4.0	5.0	6.0	7.0	8.0	9.0	10.0	11.0	12.0	
PV FCF ($MM)	-	-	-	-	(238.1)	(157.9)	(109.0)	(3.7)	84.5	166.2	229.3	258.6	245.3	216.2	193.1	187.2	181.9	2,319.7

Sum NPV FCF ($MM)	3,573	$ 21.42
Net Cash 4Q17	108.6	$ 0.65
Implied FOLD Mkt Cap ($MM)	**3,682**	
FOLD Per Share Value	**22**	

Cost of Equity	10%
Terminal Growth Rate	2%
Diluted Shares Outstanding 4Q17	166.9

Source: Company filings, Leerink Partners Research

FOLD Expected Events	
US migalastat monotherapy in Fabry	
3Q18	Possible FDA approval (PDUFA date of Aug 13)
2019	Possible US Launch
ROW migalastat (Galafold) monotherapy in Fabry	
2018	EU commercial reimbursement and EAP in addt'l territories
1H18	Regulatory decision in Japan
ERT (ATB101) + chaperone (migalastat) combo in Fabry	
2019	Initiate clinical study
ATB200 + chaperone combo (AT2221)	
1H18	Meetings with US and EU regulators
2Q18	Update on regulatory path forward
2018	Initiate Phase 3 pivotal study

Source: Company Reports, Leerink Partners Research

Disclosures Appendix

Analyst Certification

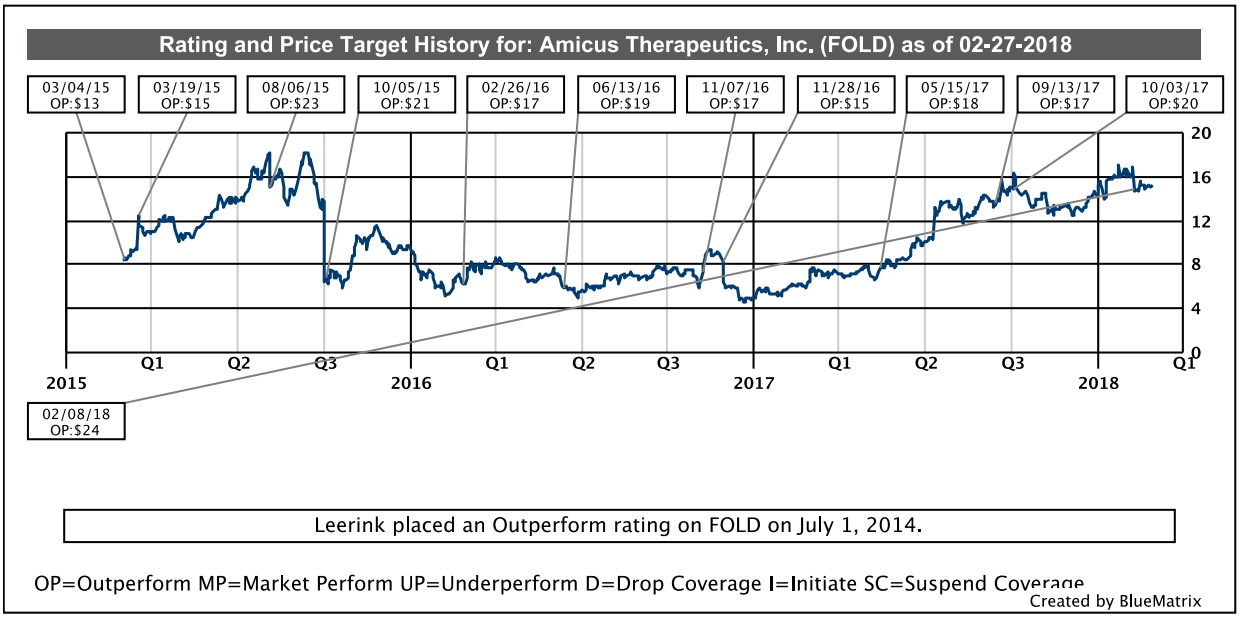
I, Joseph P. Schwartz, certify that the views expressed in this report accurately reflect my views and that no part of my compensation was, is, or will be directly related to the specific recommendation or views contained in this report.

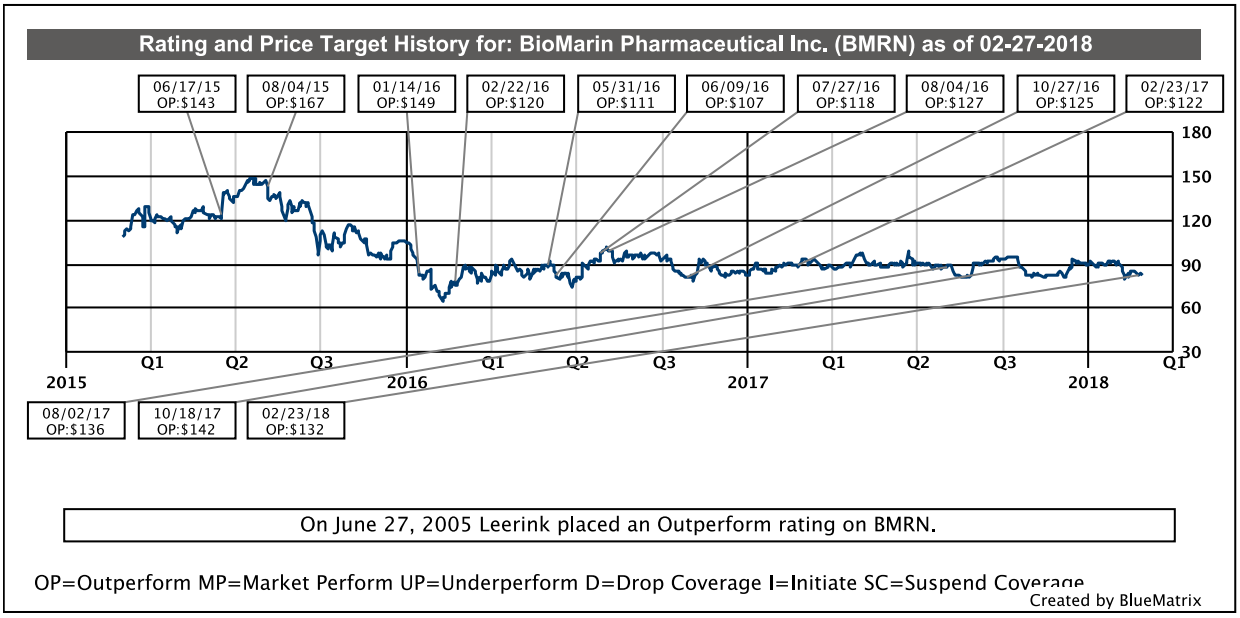
Valuation

Our price target in 12 months is $22/share and is based on a discounted cash flow (DCF) analysis of probability-weighted sales using a 10% discount rate, 2% terminal growth rate, plus net cash.

Risks to Valuation

Risks include the potential for disappointing clinical data, regulatory setbacks, commercial shortfalls, and dilutive financing. Since FOLD presently has two late-stage product candidates, any of those possible setbacks may impact the stock significantly.




Rating and Price Target History for: BioMarin Pharmaceutical Inc. (BMRN) as of 02-27-2018
06/17/15 OP:$143
08/04/15 OP:$167
01/14/16 OP:$149
02/22/16 OP:$120
05/31/16 OP:$111
06/09/16 OP:$107
07/27/16 OP:$118
08/04/16 OP:$127
10/27/16 OP:$125
02/23/17 OP:$122
180
150
120
90
60
30
Q1 Q2 Q3 Q1 Q2 Q3 Q1 Q2 Q3 Q1
2015
2016
2017
2018
08/02/17 OP:$136
10/18/17 OP:$142
02/23/18 OP:$132
On June 27, 2005 Leerink placed an Outperform rating on BMRN.
OP=Outperform MP=Market Perform UP=Underperform D=Drop Coverage I=Initiate SC=Suspend Coverage
Created by BlueMatrix

Distribution of Ratings/Investment Banking Services (IB) as of 12/31/17				
			IB Serv./Past 12 Mos.	
Rating	Count	Percent	Count	Percent
BUY [OP]	130	69.9	48	36.9
HOLD [MP]	56	30.1	2	3.6
SELL [UP]	0	0.0	0	0.0

Explanation of Ratings

Outperform (Buy): We expect this stock to outperform its benchmark over the next 12 months.

Market Perform (Hold/Neutral): We expect this stock to perform in line with its benchmark over the next 12 months.

Underperform (Sell): We expect this stock to underperform its benchmark over the next 12 months.

The degree of outperformance or underperformance required to warrant an Outperform or an Underperform rating should be commensurate with the risk profile of the company.

For the purposes of these definitions the relevant benchmark will be the S&P 600® Health Care Index for issuers with a market capitalization of less than $2 billion and the S&P 500® Health Care Index for issuers with a market capitalization over $2 billion.

Important Disclosures

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MEDACorp is a network of healthcare professionals, attorneys, physicians, key opinion leaders and other specialists accessed by Leerink and it provides information used by its analysts in preparing research.

In the past 12 months, the Firm has received compensation for providing investment banking services to Amicus Therapeutics, Inc. .

Leerink Partners LLC makes a market in Amicus Therapeutics, Inc. and BioMarin Pharmaceutical Inc.

Leerink Partners LLC has acted as a manager for a public offering of Amicus Therapeutics, Inc. in the past 12 months.

On June 27, 2005 Leerink placed an Outperform rating on BMRN.

Leerink placed an Outperform rating on FOLD on July 1, 2014.

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LEERINK

LEERINK PARTNERS LLC EQUITY RESEARCH

Director of Equity Research	**John L. Sullivan, CFA**	(617) 918-4875	john.sullivan@leerink.com
Associate Director of Research	**James Kelly**	(212) 277-6096	jim.kelly@leerink.com
Vice President	Jean Roberts, Ph.D.	(212) 277-6093	jean.roberts@leerink.com
Director of Therapeutic Research	**Geoffrey C. Porges, MBBS**	(212) 277-6092	geoffrey.porges@leerink.com
Major Pharmaceuticals	**Seamus Fernandez**	(617) 918-4011	seamus.fernandez@leerink.com
	Richard Goss	(617) 918-4059	richard.goss@leerink.com
	Etzer Darout, Ph.D.	(617) 918-4020	etzer.darout@leerink.com
Large Cap Biotechnology	**Geoffrey C. Porges, MBBS**	(212) 277-6092	geoffrey.porges@leerink.com
	Bradley Canino, CPA	(212) 277-6158	bradley.canino@leerink.com
Mid- and Small-Cap Biotechnology	**Joseph P. Schwartz**	(617) 918-4575	joseph.schwartz@leerink.com
	Michael Schmidt, Ph.D.	(617) 918-4588	michael.schmidt@leerink.com
	Paul Matteis	(617) 918-4585	paul.matties@leerink.com
	Benjamin Burnett, Ph.D.	(212) 277-6071	benjamin.burnett@leerink.com
	Jonathan Chang, Ph.D., CFA	(617) 918-4015	jonathan.chang@leerink.com
	Dae Gon Ha, Ph.D.	(617) 918-4093	daegon.ha@leerink.com
	Varun Kumar, Ph.D.	(617) 918-4518	varun.kumar@leerink.com
	Jeffrey Lin, Ph.D.	(617) 918-4838	jeffrey.lin@leerink.com
Specialty Pharmaceuticals & Generics	**Ami Fadia**	(212) 277-6047	ami.fadia@leerink.com
Life Science Tools & Diagnostics	**Puneet Souda**	(212) 277-6091	puneet.souda@leerink.com
Medical Devices, Cardiology	**Danielle Antalffy**	(212) 277-6044	danielle.antalffy@leerink.com
	Rebecca Wang	(212) 277-6087	rebecca.wang@leerink.com
	Dylan J. Gantley	(212) 277-6095	dylan.gantley@leerink.com
Medical Devices, Orthopedics	**Richard Newitter**	(212) 277-6088	richard.newitter@leerink.com
	Jaime L. Morgan	(212) 277-6073	jaime.morgan@leerink.com
	Dylan J. Gantley	(212) 277-6095	dylan.gantley@leerink.com
Healthcare Services, Managed Care & Facilities	**Ana Gupte, Ph.D.**	(212) 277-6040	ana.gupte@leerink.com
	John Sourbeer	(212) 277-6139	john.sourbeer@leerink.com
Healthcare Technology & Distribution, Digital Health	**David Larsen, CFA**	(617) 918-4502	david.larsen@leerink.com
	Jonathan McGraw Bentley	(617) 918-4887	jonathan.bentley@leerink.com
	Matt Dellelo, CFA	(617) 918-4812	matt.dellelo@leerink.com
Sr. Editor/Supervisory Analyst	Mary Ellen Eagan, CFA	(617) 918-4837	maryellen.eagan@leerink.com
Supervisory Analysts	Randy Brougher		randy.brougher@leerink.com
	Robert Egan		bob.egan@leerink.com
	Amy N. Sonne		amy.sonne@leerink.com
Editorial Associate	Emily Singletary	(212) 277-6115	emily.singletary@leerink.com

BOSTON
One Federal St., 37th Fl.
Boston, MA 02110
(800) 808-7525

NEW YORK
299 Park Ave., 21st Fl.
New York, NY 10171
(800) 778-1653

CHARLOTTE
227 West Trade St., Ste. 2050
Charlotte, NC 28202
(704) 969-8944

SAN FRANCISCO
255 California St., 12th Fl.
San Francisco, CA 94111
(415) 905-7200