

## BioMarin Pharma (BMRN)

**\$85.22** (As of 04/16/20)

Price Target (6-12 Months): **\$90.00**

Long Term: 6-12 Months

**Zacks Recommendation:**

**Neutral**

(Since: 01/15/20)

Prior Recommendation: Outperform

Short Term: 1-3 Months

**Zacks Rank:** (1-5)

**3-Hold**

Zacks Style Scores:

VGM:B

Value: D

Growth: A

Momentum: B

### Summary

Sales of BioMarin's key drugs — Vimizim and Kuvan — are being driven by strong demand trends. Its newest product, Palynziq is witnessing strong commercial uptake in the United States. BioMarin's rare disease pipeline is progressing well with multiple catalysts in 2020. The company filed regulatory filings for valrox in late 2019 and targets the same for vosoritide in 2020. However, any regulatory setbacks related to valrox and vosoritide can hurt the stock. Moreover, Kuvan is expected to face generic competition in the fourth quarter of 2020, which can hurt sales. The stock has outperformed the industry this year so far. Estimates have gone down slightly ahead of Q1 earnings due to coronavirus uncertainty. BioMarin has a mixed record of earnings surprises in the recent quarters.

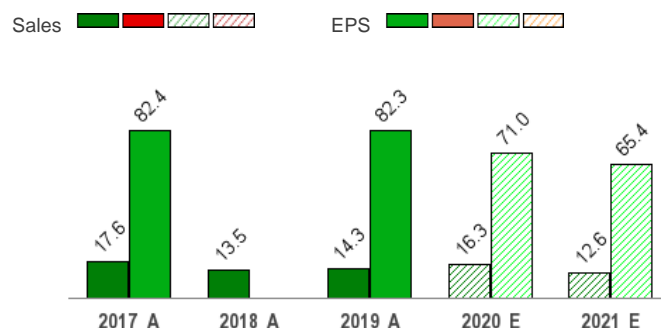
### Price, Consensus & Surprise



### Data Overview

52 Week High-Low	\$97.10 - \$62.88
20 Day Average Volume (sh)	1,574,234
Market Cap	\$15.4 B
YTD Price Change	0.8%
Beta	1.05
Dividend / Div Yld	\$0.00 / 0.0%
Industry	<a href="#">Medical - Biomedical and Genetics</a>
Zacks Industry Rank	Top 8% (20 out of 253)

### Sales and EPS Growth Rates (Y/Y %)



Last EPS Surprise	19.1%
Last Sales Surprise	-1.6%
EPS F1 Est- 4 week change	0.0%
Expected Report Date	04/30/2020
Earnings ESP	0.0%
P/E TTM	93.7
P/E F1	53.6
PEG F1	1.0
P/S TTM	9.0

### Sales Estimates (millions of \$)

	Q1	Q2	Q3	Q4	Annual*
2021	534 E	530 E	553 E	580 E	2,231 E
2020	469 E	478 E	502 E	532 E	1,982 E
2019	401 A	388 A	461 A	454 A	1,704 A

### EPS Estimates

	Q1	Q2	Q3	Q4	Annual*
2021	\$0.58 E	\$0.50 E	\$0.49 E	\$0.62 E	\$2.63 E
2020	\$0.33 E	\$0.34 E	\$0.43 E	\$0.51 E	\$1.59 E
2019	\$0.14 A	\$0.09 A	\$0.43 A	\$0.25 A	\$0.93 A

\*Quarterly figures may not add up to annual.

The data in the charts and tables, including the Zacks Consensus EPS and Sales estimates, is as of 04/16/2020. The reports text is as of 04/17/2020.

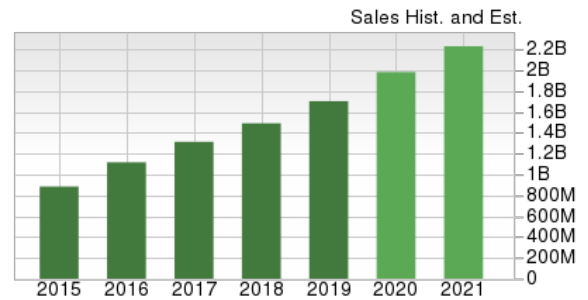
## Overview

San Rafael, CA-based BioMarin Pharmaceutical Inc. focuses on the development and commercialization of treatments for serious life threatening medical conditions, mostly for children. The company's portfolio comprises seven marketed products namely Aldurazyme (mucopolysaccharidosis type I (MPS I)), Naglazyme (MPS VI), Kuvan (phenylketonuria (PKU) – a rare genetic enzyme deficiency disorder), Vimizim (MPS IVA or Morquio syndrome type A), Brineura (CLN2– a form of Batten disease) and Palynziq (PKU). BioMarin has a collaboration agreement with Sanofi for Aldurazyme. BioMarin receives royalties on Aldurazyme sales from Genzyme

Interesting candidates in BioMarin's pipeline include valoctocogene roxaparvovec (hemophilia A) and vosoritide (achondroplasia) among others.

In Jan 2015, BioMarin acquired a Dutch biotech company Prosensa, in an all-cash transaction valued at \$751.5 million, to boost its rare disease pipeline. Moreover, the company acquired all global rights to Kuvan, excluding Japan, and pegvaliase from Merck Serono in Jan 2016, for cash payments of \$374.5 million plus certain milestone payments.

BioMarin generated total sales of \$1.7 billion in 2019, up 14%.



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## Reasons To Buy:

- ▲ **Share Price Outperforming Industry:** BioMarin's share price movement shows that the stock has outperformed the industry this year so far. The stock has risen 0.7% during this period, outperforming 2.2% decrease for the industry.
- ▲ **Key Products Continue to Drive Growth:** Vimizim is doing well, surpassing the company's expectations. Vimizim's sales rose 17% in 2018 and 13% in 2019. Other products like Kuvan are also performing well. Kuvan's North American sales continue to be propelled by growth in new patients and high levels of adherence. Internationally, uptake has been solid and the acquisition of the drug's global rights has opened a stream of orders directly from the majority of the top markets to the company. Kuvan's sales rose 7% in 2019.

BioMarin's key drugs, Vimizim and Kuvan, enjoy strong demand trends. It has an impressive rare disease pipeline with a growing focus towards gene therapy agents.

Brineura was approved for the treatment of children with CLN2 disease – a form of Batten disease – both in the United States and the EU in 2017. Brineura sales were \$72 million in 2019 and are expected to be much higher, in the range of \$85-\$115 million, in 2020.

Meanwhile, Palynziq (pegvaliase) injection, developed to reduce blood phenylalanine (Phe) levels in PKU patients, gained FDA approval in May 2018 and was launched in July 2018. In the EU, a marketing application was approved in May 2019 with meaningful revenue contributions from this region expected in 2020. Palynziq is witnessing strong commercial uptake in the United States. BioMarin believes the injection has peak commercial opportunity of roughly \$1 billion as it demonstrated dramatic Phe reductions in PKU patients in pivotal studies. BioMarin is optimistic that pegvaliase will be the approved treatment of choice for adults, complementing Kuvan, which is a popular treatment choice for children. Palynziq sales were \$87 million in 2019 and are expected to be much higher, in the range of \$180-\$210 million, in 2020.

BioMarin believes it can generate approximately \$2 billion in total revenues in 2020 on the back of its currently commercialized seven products with sales accelerating thereafter through the potential approval and launch of valrox and vosoritide.

- ▲ **Promising Pipeline:** We are pleased with BioMarin's efforts to build its orphan disease-focused pipeline. The company has a robust rare disease pipeline with several data readouts lined up in the coming quarters.

BioMarin's regulatory applications for valoctocogene roxaparvovec (valrox), a gene therapy for severe hemophilia A, are under review in the United States (PDUFA Date: Aug 21) and Europe with potential approval and launch in the second half of 2020. BioMarin conducted two separate phase III studies — GENE8-1 (6e13 vg/kg dose) and GENE8-2 (4e13 vg/kg dose) — on valrox for the treatment of patients without the pre-existing AAV5 antibodies. The interim phase III data from the GENE8-1 study and updated three-year data from a long-term phase I/II study on valrox formed the basis of the regulatory submissions. Valrox is anticipated to be transformational, if approved, as it has the potential to dramatically change the treatment paradigm.

Another important candidate in its pipeline is vosoritide, which is in a phase II study in children aged 5 to 14 with achondroplasia, the most common form of dwarfism. BioMarin estimates that around 25,000 children suffer from this disorder in its commercial territories, which represents decent sales growth opportunity. Data from this ongoing study has shown 9 centimeters of cumulative additional height gained at 54 months.

Meanwhile a 52-week phase III pivotal study (n~110) evaluated vosoritide in children (ages 5-14) with achondroplasia. Top-line data from this study showed that patients treated with the candidate achieved placebo-adjusted change from baseline in growth velocity of 1.6 cm/year after treatment duration of one year. The company is planning to file regulatory applications for vosoritide in United States and EU in the third quarter of 2020 based on data from the above pivotal study as well as long-term safety and efficacy data from the phase II study and ongoing extension studies.

In addition, BioMarin initiated a separate phase II study in 2018 to evaluate the effect of vosoritide in infants and young children (less than 60 months old). We note that vosoritide has Orphan Drug status in both the United States and the EU. Also, BioMarin plans to begin phase I/II study on vosoritide for a second indication — dominantly inherited short stature (DISS) — later in 2020, as part of a research collaboration with Children's National Hospital.

A third PKU treatment option in BioMarin's PKU franchise is its gene therapy candidate, BMN 307. Phase I/II study on BMN 307 is expected to begin dosing in the first quarter of 2020.

Successful development and commercialization of these candidates will help drive long-term growth at BioMarin.

- ▲ **Deals to Boost Portfolio and Strengthen Pipeline:** In January 2016, BioMarin acquired all global rights to Kuvan and pegvaliase (now marketed as Palynziq) from Merck Serono. Previously, the company had exclusive rights to Kuvan in the U.S. and Canada and to pegvaliase in the U.S. and Japan. With this transaction, BioMarin has gained exclusive worldwide rights to Kuvan and pegvaliase with the exception of Kuvan in Japan. The acquisition of PKU rights in these markets represents significant opportunity for BioMarin to establish commercial leadership for the treatment of PKU, first with Kuvan, and then with pegvaliase.

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## Reasons To Sell:

▼ **Pipeline Setbacks:** While we believe that BioMarin has an impressive pipeline, delayed approvals or development setbacks would have a negative impact on the stock. In the second quarter of 2016, BioMarin discontinued clinical and regulatory development of Kyndrisa for the treatment of Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping. The decision followed the company's discussions with the European Medicines Agency's Committee for Medicinal Products for Human Use, which hinted at the issuance of a negative opinion. The FDA had also issued a complete response letter in January 2016 for Kyndrisa stating that the standard of substantial evidence of Kyndrisa effectiveness was not met.

BioMarin has also discontinued the development of the three follow-on products of Kyndrisa – BMN 044, BMN 045, and BMN 053 – that were being evaluated in phase II studies for specific forms of DMD. BioMarin terminated internal development of the reveglucosidase alfa program (for the treatment of patients with late-onset Pompe disease). Also, BioMarin halted further development of BMN-195 for a type of muscle disease following disappointing results from an early-stage study.

▼ **Kuvan Faces Generic Threat:** BioMarin is facing generic threat for Kuvan, one of its most promising drugs, which has been performing well since its launch. Per settlements with Dr Reddy's and Par Pharmaceuticals, Kuvan generics are expected to enter the U.S. market from October 2020.

Generic challenges for Kuvan are concerning. Additional setbacks on the development/regulatory front could pull down the stock significantly.

## Last Earnings Report

### BioMarin Q4 Earnings Beat, Sales Miss

BioMarin's adjusted earnings of 25 cents per share beat the Zacks Consensus Estimate of 21 cents. In the year-ago quarter, the company had incurred loss of 6 cents per share.

Total revenues were \$454.4 million in the reported quarter, up 29% from the year-ago period, driven by higher product revenues. Sales missed the Zacks Consensus Estimate of \$462 million.

Quarter Ending **12/2019**

Report Date	Feb 26, 2020
Sales Surprise	-1.61%
EPS Surprise	19.05%
Quarterly EPS	0.25
Annual EPS (TTM)	0.91

### Quarterly Details

Product revenues (including Aldurazyme) were \$436.6 million in the fourth quarter, reflecting a 25.7% increase year over year. Product revenues from BioMarin's marketed brands (excluding Aldurazyme) grew 25% year over year to \$412.7 million. Royalty and other revenues were \$17.9 million in the quarter, higher than \$6.0 million a year ago.

Its PKU franchise sales rose 28% year over year in the quarter to \$154.3 million. Kuvan revenues rose 9% to \$122.6 million driven by patient growth in North America.

Palynziq injection sales grossed \$31.7 million in the quarter compared with \$24.1 million in the previous quarter, driven by new patients initiating therapy as well as the growing number of patients who have now achieved maintenance dosing. The majority of Palynziq sales came from the United States. As of Dec 30, 2019, 762 U.S. commercial patients were on treatment with Palynziq versus 670 at the end of the third quarter. Of the 762 patients, 137 were from clinical studies and 625 were naive to Palynziq treatment. Another 143 patients have been enrolled and are awaiting their first treatment with Palynziq.

In the EU, Palynziq injection was approved in May. BioMarin said that multiple clinics across Germany were now treating patients. The company is working to get reimbursement approvals in other European countries and expects meaningful contribution from European sales of Palynziq in 2020.

Naglazyme sales rose 24% to \$94.8 million mainly due to orders from Brazil.

Vimizim contributed \$132.3 million to total revenues, up 16% year over year owing to patient growth in the United States and orders from Brazil.

Naglazyme and Vimzim revenues vary on a quarterly basis, primarily due to the timing of central government orders from some countries, mainly Brazil.

Brineura generated sales of \$25.2 million in the fourth quarter, higher than \$19.8 million reported in the previous quarter due to patient growth.

BioMarin received Aldurazyme royalties totaling \$23.9 million from Genzyme in the quarter, up 37% year over year.

R&D expenses rose 3.9% year over year to \$155.5 million due to costs for pipeline development. Marketing expenses associated with Palynziq commercial efforts and launch preparations for valrox resulted in 15.6% increase in SG&A expenses to \$161.2 million.

As of Dec 31, 2019, BioMarin had \$1.2 billion in cash, cash equivalents and investments, same as at the end of Sep 30, 2019.

### 2019 Results

Full-year 2019 sales rose 14% to \$1.70 billion, slightly missing the Zacks Consensus Estimate of \$1.71 billion. However, sales were within the guided range of \$1.69-\$1.72 billion.

Adjusted earnings of 93 cents per share beat the Zacks Consensus Estimate of 89 cents. Earnings rose 82% year over year.

### 2020 Guidance

BioMarin expects total revenues in 2020 to come in the range of \$1.95-\$2.05 billion, which indicates growth of 17% at the middle of the range. Vimizim sales are expected in the range of \$560-\$610 million for the full year. Kuvan sales are projected within \$430-\$480 million. Naglazyme sales are predicted in the band of \$380-\$420 million. Brineura sales are expected within \$85-\$115 million. Palynziq sales are forecast in the \$180-\$210 million band.

R&D costs are expected to be within \$675-\$725 million. SG&A expenses are anticipated in the range of \$780-\$830 million.

The company expects adjusted net income in the range of \$260-\$310 million, the midpoint of which indicates growth of more than 70%.

## Recent News

### To File Regulatory Applications for Vosoritide in Q3 – Apr 6

BioMarin announced that it is planning to file regulatory applications for vosoritide in the third quarter in the United States and Europe following its meetings with health authorities. The regulatory applications will be filed based on data from successfully completed phase III study, which evaluated vosoritide as a treatment for achondroplasia in children aged 5 to 14 for 52 weeks. The filings will be further supported by long-term safety and efficacy from a phase II study and ongoing extension studies on vosoritide in dwarf children.

A potential approval will make the drug the first and only approved treatment for achondroplasia in the United States and Europe.

### FDA Grants Priority Review to Valrox BLA –Feb 20

BioMarin announced that the FDA has accepted and granted priority review to its biologics license application (BLA) seeking approval for valoctocogene roxaparvovec. With the FDA granting priority review to the application, a decision is expected on August 21. BioMarin said that the FDA has informed that no advisory committee meeting is currently planned to review the application. Per the company, if the BLA is approved, valoctocogene roxaparvovec will be the first gene therapy in U.S. to be approved to treat any type of hemophilia.

In addition, the FDA has also accepted a premarket approval application for a companion diagnostic test for valoctocogene roxaparvovec. Meanwhile, the EMA has also validated the marketing application for valoctocogene roxaparvovec. BioMarin said that the EMA's review process commenced in January under accelerated assessment.

## Valuation

BioMarin's shares are up 0.7% in the year-to-date period and 2.5% over the trailing 12-month period. Stocks in the Zacks sub-industry and sector are down 2.2% and 6.3%, respectively in the year-to-date period. Over the past year, the Zacks sub-industry and sector are up 2.2% and 1.8%, respectively.

The S&P 500 Index is down 13.2% in the year-to-date period and 4.0% in the past year.

The stock is currently trading at 9.12X trailing 12-month sales per share, which compares to 3.13X for the Zacks sub-industry, 2.93X for the Zacks sector and 3.02X for the S&P 500 Index.

Over the past five years, the stock has traded as high as 27.84X and as low as 7.65X, with a 5-year median of 12.62X. Our Neutral recommendation indicates that the stock will perform in-line with the market. Our \$90 price target reflects 9.6X forward 12-month sales per share.

The table below shows summary valuation data for BMRN

Valuation Multiples - BMRN					
		Stock	Sub-Industry	Sector	S&P 500
P/S TTM	Current	9.12	3.13	2.93	3.02
	5-Year High	27.84	4.62	4.16	3.69
	5-Year Low	7.65	2.19	2.39	2.5
	5-Year Median	12.62	2.67	3.26	3.19
P/B TTM	Current	4.91	3.81	3.62	3.71
	5-Year High	12.44	5.41	5.04	4.54
	5-Year Low	4	2.48	3.01	2.9
	5-Year Median	5.68	3.33	4.29	3.64

As of 4/16/2020

## Industry Analysis Zacks Industry Rank: Top 8% (20 out of 253)



## Top Peers

Senesco Technologies Inc. (ELOX)	Outperform
Ascendis Pharma A/S (ASND)	Neutral
Editas Medicine, Inc. (EDIT)	Neutral
Amicus Therapeutics, Inc. (FOLD)	Neutral
Ultragenyx Pharmaceutical Inc. (RARE)	Neutral
Reata Pharmaceuticals, Inc. (RETA)	Neutral
REGENXBIO Inc. (RGNX)	Neutral
Sangamo Therapeutics, Inc. (SGMO)	Neutral

Industry Comparison Industry: Medical - Biomedical And Genetics				Industry Peers		
	BMRN Neutral	X Industry	S&P 500	ASND Neutral	ELOX Outperform	RETA Neutral
<b>VGM Score</b>	<b>B</b>	-	-	<b>F</b>	<b>C</b>	<b>F</b>
Market Cap	15.40 B	171.04 M	19.06 B	6.14 B	78.85 M	4.68 B
# of Analysts	11	3	14	7	3	6
Dividend Yield	0.00%	0.00%	2.26%	0.00%	0.00%	0.00%
<b>Value Score</b>	<b>D</b>	-	-	<b>F</b>	<b>C</b>	<b>F</b>
Cash/Price	0.05	0.26	0.06	0.12	0.64	0.14
EV/EBITDA	473.78	-2.58	11.49	-23.36	-0.67	-14.47
PEG Ratio	1.03	1.71	2.09	NA	NA	NA
Price/Book (P/B)	4.90	3.10	2.55	8.10	2.17	18.18
Price/Cash Flow (P/CF)	133.75	14.50	10.06	NA	NA	NA
P/E (F1)	54.18	28.34	17.56	NA	NA	NA
Price/Sales (P/S)	9.04	13.42	1.94	408.44	NA	176.32
Earnings Yield	1.87%	-18.70%	5.57%	-4.43%	-51.78%	-5.86%
Debt/Equity	0.16	0.02	0.70	0.05	0.30	0.60
Cash Flow (\$/share)	0.64	-1.04	7.01	-5.62	-1.25	-8.67
<b>Growth Score</b>	<b>A</b>	-	-	<b>F</b>	<b>D</b>	<b>F</b>
Hist. EPS Growth (3-5 yrs)	NA%	18.12%	10.92%	NA	NA	NA
Proj. EPS Growth (F1/F0)	71.45%	4.64%	-3.36%	-8.57%	23.63%	13.54%
Curr. Cash Flow Growth	200.25%	13.18%	5.93%	55.11%	6.63%	268.02%
Hist. Cash Flow Growth (3-5 yrs)	27.84%	8.03%	8.55%	NA	NA	NA
Current Ratio	2.08	4.72	1.24	12.73	4.89	3.49
Debt/Capital	13.47%	4.35%	42.78%	4.89%	23.18%	37.64%
Net Margin	-1.40%	-229.34%	11.64%	-1,623.79%	NA	-1,094.28%
Return on Equity	0.81%	-65.95%	16.74%	-32.80%	-118.94%	-766.88%
Sales/Assets	0.38	0.20	0.54	0.02	NA	0.07
Proj. Sales Growth (F1/F0)	16.33%	5.91%	-0.14%	-31.59%	NA	-56.00%
<b>Momentum Score</b>	<b>B</b>	-	-	<b>B</b>	<b>A</b>	<b>A</b>
Daily Price Chg	3.47%	0.00%	-0.20%	1.30%	-4.61%	-1.60%
1 Week Price Chg	-5.53%	10.01%	16.01%	8.36%	41.03%	9.57%
4 Week Price Chg	14.73%	20.61%	14.56%	27.26%	-23.24%	1.04%
12 Week Price Chg	-0.48%	-18.74%	-22.94%	-6.15%	-56.43%	-31.97%
52 Week Price Chg	2.48%	-27.40%	-15.02%	19.33%	-82.50%	79.20%
20 Day Average Volume	1,574,234	227,526	3,220,598	210,111	110,166	349,160
(F1) EPS Est 1 week change	0.00%	0.00%	0.00%	0.00%	0.00%	0.00%
(F1) EPS Est 4 week change	0.00%	0.00%	-7.09%	-14.56%	0.00%	2.62%
(F1) EPS Est 12 week change	-29.85%	-1.01%	-9.32%	-14.56%	29.67%	-19.51%
(Q1) EPS Est Mthly Chg	0.00%	0.00%	-10.68%	-15.37%	0.00%	1.04%

## Zacks Style Scores

The Zacks Style Score is as a complementary indicator to the Zacks rating system, giving investors a way to focus on the highest rated stocks that best fit their own stock picking preferences.

Academic research has proven that stocks with the best Value, Growth and Momentum characteristics outperform the market. The Zacks Style Scores rate stocks on each of these individual styles and assigns a rating of A, B, C, D and F. We also produce the VGM Score (V for Value, G for Growth and M for Momentum), which combines the weighted average of the individual Style Scores into one score. This is perfectly suited for those who want their stocks to have the best scores across the board.

Value Score	D
Growth Score	A
Momentum Score	B
VGM Score	B

As an investor, you want to buy stocks with the highest probability of success. That means buying stocks with a Zacks Recommendation of Outperform, which also has a Style Score of an A or a B.

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