BioMarin Pharma (BMRN)

$89.01 (As of 02/14/20)

Price Target (6-12 Months): **$94.00**

Long Term: 6-12 Months	**Zacks Recommendation:** (Since: 01/15/20) Prior Recommendation: Outperform	**Neutral**
Short Term: 1-3 Months	**Zacks Rank:** (1-5)	**3-Hold**
	Zacks Style Scores:	VGM:C
	Value: D | Growth: A | Momentum: F	

Summary

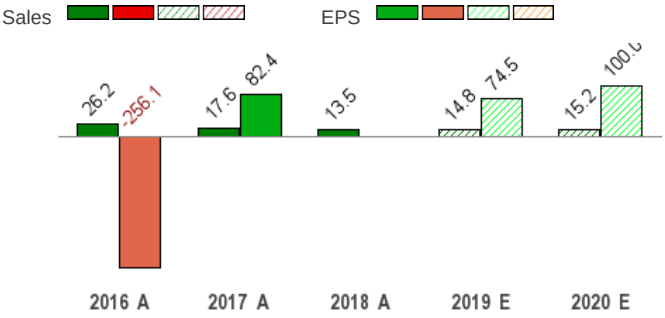
Sales of BioMarin's key orphan disease drugs — Vimizim and Kuvan — are being driven by strong demand trends. Its rare disease pipeline is also progressing well. The company filed regulatory applications for Valrox in late 2019 and targets the same for vosoritide in 2020. Growing pipeline focus on gene therapy agents is encouraging. Valrox, a gene therapy for hemophilia A, is anticipated to be transformational, if approved. However, uneven quarterly product sales of Naglazyme and Vimzin, owing to infrequent ordering patterns in some ex-U.S. countries, are a concern. The stock has underperformed the industry in the past one year. Estimates movement is mixed ahead of the company's Q4 earnings. However, BioMarin has a rather negative record of earnings surprises in the recent quarters.

Price, Consensus & Surprise

Data Overview

52 Week High-Low	**$96.20 - $62.88**
20 Day Average Volume (sh)	**1,065,504**
Market Cap	**$16.0 B**
YTD Price Change	**5.3%**
Beta	**1.39**
Dividend / Div Yld	**$0.00 / 0.0%**
Industry	**Medical - Biomedical and Genetics**
Zacks Industry Rank	**Top 28% (72 out of 255)**
Last EPS Surprise	**26.5%**
Last Sales Surprise	**1.2%**
EPS F1 Est- 4 week change	**-0.2%**
Expected Report Date	**02/26/2020**
Earnings ESP	**-14.6%**
P/E TTM	**150.9**
P/E F1	**50.0**
PEG F1	**1.0**
P/S TTM	**10.0**

Sales and EPS Growth Rates (Y/Y %)



Sales Estimates (millions of $)

	Q1	Q2	Q3	Q4	Annual*
2020	469 E	478 E	492 E	502 E	1,973 E
2019	401 A	388 A	461 A	462 E	1,712 E
2018	373 A	373 A	392 A	353 A	1,491 A

EPS Estimates

	Q1	Q2	Q3	Q4	Annual*
2020	$0.36 E	$0.41 E	$0.49 E	$0.26 E	$1.78 E
2019	$0.14 A	$0.09 A	$0.43 A	$0.22 E	$0.89 E
2018	$0.11 A	$0.12 A	$0.14 A	-$0.07 A	$0.51 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 02/14/2020. The reports text is as of 02/17/2020.

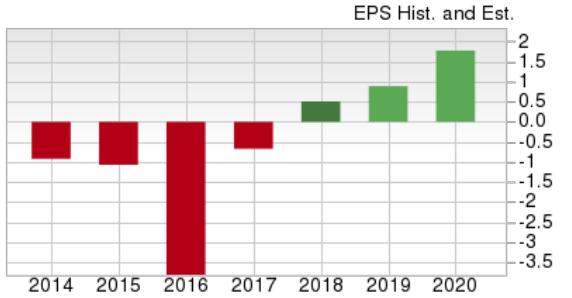
Overview

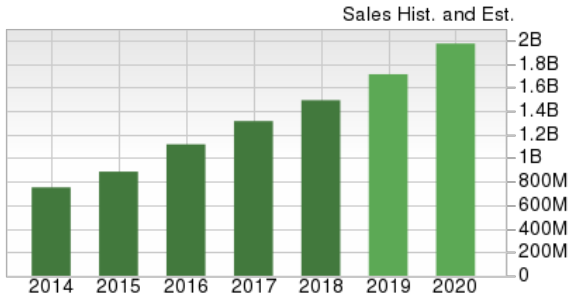
San Rafael, CA-based BioMarin Pharmaceutical Inc. focuses on the development and commercialization of treatments for serious life threatening medical conditions, mostly for children. The company's portfolio comprises seven marketed products namely Aldurazyme (mucopolysaccharidosis type I (MPS I)), Naglazyme (MPS VI), Kuvan (phenylketonuria (PKU) – a rare genetic enzyme deficiency disorder), Firdapse (Lambert Eaton myasthenic syndrome (LEMS) – a rare autoimmune 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 $1.5 billion in 2018, up 14%.





Reasons To Buy:

> 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.

- **Key Products Continue to Drive Growth:** Vimizim's approval in 2014 was a major catalyst for BioMarin. Vimizim is doing well, surpassing the company's expectations. Vimizim's sales rose 17% in 2018 and are expected in the range of $540 million to $570 million 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 sales are projected in the range of $455–$475 million in 2019.

  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 are expected in the range of $55-$75 million in 2019.

  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 in 2020. It 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 are expected in the range of $80-$10 million in 2019.

  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 as its late stage pipeline products reach commercialization.

- **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.

  A key pipeline candidate is valoctocogene roxaparvovec (valrox), a gene therapy for severe hemophilia A. BioMarin conducted two separate phase III studies — GENEr8–1 (6e13 vg/kg dose) and GENEr8–2 (4e13 vg/kg dose) — on valrox for the treatment of patients without the pre-existing AAV5 antibodies. BioMarin filed regulatory applications for valrox in U.S/EU in late 2019 with potential approval and launch in the second half of 2020. The interim phase III data from the GENEr8-1 study and updated three-year data from a long-term phase I/II study on valrox formed the basis of these regulatory submissions.

  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 5.7 centimeters of cumulative additional height gained at 42 months.

  Meanwhile a 52-week phase III study (n~110) is also ongoing for the treatment of 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/yr after a treatment duration of one year. This will be followed by an open-label extension study with the lower dose (15 µg/kg/day). 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.

  A potential third PKU treatment option in BioMarin's PKU franchise is its gene therapy candidate, BMN 307, which expected to move into clinical studies in 2020.

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

- **Deals to Boost Portfolio and Strengthen Pipeline: I**n January 2016, BioMarin acquired all global rights to Kuvan and pegvaliase 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.

Reasons To Sell:

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

- **Share Price Underperforming Industry:** BioMarin's share price movement shows that the stock has underperformed the industry in the past year. The stock has declined 5.3% during this period against 4.9% decrease for the industry.

- **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.

Last Earnings Report

BioMarin Q3 Earnings & Sales Beat

Quarter Ending	09/2019
Report Date	**Oct 23, 2019**
Sales Surprise	**1.17%**
EPS Surprise	**26.47%**
Quarterly EPS	**0.43**
Annual EPS (TTM)	**0.59**

BioMarin announced better-than-expected earnings and revenues for the third quarter of 2019.

Adjusted earnings of 43 cents per share beat the Zacks Consensus Estimate of 34 cents and came ahead of the year-ago quarterly earnings of 34 cents. Earnings increased on higher product revenues and R&D expense management.

Total revenues were $461.1 million in the reported quarter, up 18% from the year-ago period, driven by higher product revenues. Sales too beat the Zacks Consensus Estimate of $456 million.

Quarterly Details

Product revenues (including Aldurazyme) were $450.9 million in the third quarter, reflecting a 16.7% increase year over year, driven by higher sales of Vimizim and newest brands, Palyzniq and Brineura. Product revenues from BioMarin's marketed brands (excluding Aldurazyme) grew 19% year over year to $428.1 million. Royalty and other revenues were $10.2 million in the quarter, higher than $5.4 million a year ago.

Its PKU franchise sales rose 23% year over year in the quarter to $144.7 million. Kuvan revenues rose 6% to $120.6 million driven by patient growth in North America.

Palynziq injection sales grossed $24.1 million in the third quarter compared with $18.8 million in the previous quarter, driven by new patients initiating therapy as well as the growing number of patients who have now achieved once-daily dosing. The majority of Palynziq sales came from the United States. As of Sep 30, 2019, 670 U.S. commercial patients were on treatment with Palynziq versus 551 at the end of the second quarter. Of the 670 patients, 142 were from clinical studies and 528 were naive to Palynziq treatment. Another 153 patients have been enrolled and are awaiting their first treatment with Palynziq. Regarding the EU launch, where Palynziq injection was approved in May, BioMarin said that its physicians in Germany now treating patients. The company said that it will take time to realize material revenue contributions from Europe and does not expect meaningful contribution from European sales of Palynziq until 2020.

Naglazyme sales decreased 8% to $94.4 million as higher revenues from Brazil were offset by lower volumes due to unfavorable government ordering patterns from certain Latin American and European countries. Vimizim contributed $163.5 million to total revenues, up 33% year over year owing to increased sales volume on the back of government orders in certain Middle Eastern countries and a large order from Brazil. Net global patient growth was 11% year over year. 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 $19.8 million in the third quarter, higher than $14.8 million reported in the previous quarter due to patient growth.

BioMarin received Aldurazyme royalties — totaling $22.8 million — from Genzyme in the quarter, down 17% year over year due to changes in revenue recognition rules.

R&D expenses rose 8.7% year over year to $152.3 million due to late-stage pipeline development. Marketing expenses associated with Palynziq commercial efforts and launch preparations for its pipeline candidate, valoctocogeneroxaparvovec or valrox resulted in 19.55% increase in SG&A expenses to $141.3 million.

As of Jun 30, 2019, BioMarin had $1.2 billion in cash, cash equivalents and investments versus $1.1 billion at the end of Jun 30, 2019.

2019 Outlook

BioMarin tightened its total revenue guidance for 2019 from a range of $1.68-$1.75 billion to $1.69-$1.72 billion. The company expects to generate revenues in the middle of this range. Total revenues are expected to be hurt by currency impact in the range of approximately $20 million to $25 million. In 2020, management expects to generate $2 billion in commercial revenues. Vimizim sales are expected in the range of $540-$570 million for the full year compared with $530-$570 million expected earlier. Kuvan sales are projected within $455-$475 million compared with $420-$460 million expected earlier. Naglazyme sales are predicted in the band of $360-$380 million compared with $350-$380 million expected earlier. Brineura sales are expected within $55-$75 million. Palynziq sales are forecast in the $80-$100 million band, mostly from the U.S. market. The prior expectation for Palynziq sales was $70-$100 million.

R&D costs are expected to be within $710-$740 million lower than the prior range of $740-$780 as it has ceased development of BMN-290 and licensed BMN-250 to Allievex. SG&A expenses are anticipated in the range of $670-$690 million compared with $650-$690 million. SG&A is expected to come in at the upper end of the guided range. The company expects adjusted net income in the range of $150-$170 million, tightened from the prior guidance of $130-$170 million. The company expects earnings to come at the higher end of the range due to continued R&D expense management.

Recent News

CFO Resigns – Feb 3

BioMarin announced that Dan Spiegelman has stepped down from his position as chief financial officer (CFO). Meanwhile, Brian R. Mueller, BioMarin's chief accounting officer and 17-year company veteran has been named acting CFO as the company initiates an internal and external search for the role of CFO. Spiegelman will assist with transition until September 1, 2020.

FDA Approves IND for BMN 307– Jan 13

BioMarin announced that the FDA has granted the company Investigational New Drug status for its gene therapy candidate, BMN 307 in patients with PKU. The company has also received approval for its Clinical Trial Application for BMN 307 permitting it to initiate a clinical study in the United Kingdom. The company is planning to initiate dosing in a phase I/II study — PHEARLESS — in the first quarter of 2020.

Files BLA for Valrox – Dec 23

BioMarin announced that it has submitted a biologics license application ("BLA") to the FDA seeking approval for valoctocogene roxaparvovec, an investigational gene therapy, developed for treating adult patients with severe hemophilia A. The company anticipates that the review of the BLA will be initiated by the regulatory authority in February 2020. The FDA will provide an update on whether it will accept the BLA or not after completing the filing review.

Announces Data from Phase III Achondroplasia Study – Dec 16

BioMarin announced positive data from the phase III study evaluating vosoritide in children aged 5 to 14 with achondroplasia. Data showed that patients treated with the candidate achieved placebo-adjusted change from baseline in growth velocity of 1.6 cm/yr after a treatment duration of one year. The company is planning to meet regulatory authorities in the first half of 2020 to discuss plans for submission of marketing applications for the candidate.

Valuation

BioMarin's shares are down 5.3% in the year-to-date period but up 5.3% over the trailing 12-month period. While stocks in the Zacks sub-industry are down 4.3%, that in the sector are up 1.9% in the year-to-date period. Over the past year, the Zacks sub-industry and sector are up 0.6% and 2.1%, respectively.

The S&P 500 Index is up 21% in the year-to-date period and 4.9% in the past year.

The stock is currently trading at 10.33X forward 12-month sales per share, which compares to 2.82X for the Zacks sub-industry, 3.19X for the Zacks sector and 4.04X for the S&P 500 Index.

Over the past five years, the stock has traded as high as 27.85X and as low as 7.46X, with a 5-year median of 12.9X. Our Neutral recommendation indicates that the stock will perform in-line with the market. Our $94 price target reflects 10.9X forward 12-month sales per share.

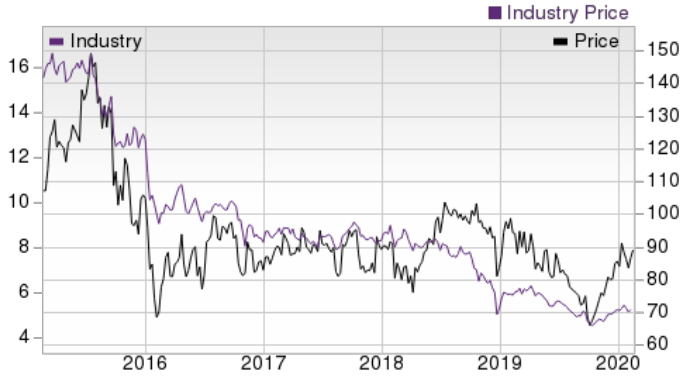
The table below shows summary valuation data for BMRN

Valuation Multiples - BMRN

		Stock	Sub-Industry	Sector	S&P 500
P/S TTM	Current	10.33	2.82	3.19	4.04
	5-Year High	27.85	5.03	4.17	4.04
	5-Year Low	7.46	2.12	2.7	2.49
	5-Year Median	12.9	2.65	3.28	3.14
P/B TTM	Current	5.2	3.88	4.63	4.36
	5-Year High	13.29	5.79	5.04	4.42
	5-Year Low	3.76	2.43	3.44	2.85
	5-Year Median	5.66	3.27	4.31	3.62

As of 2/14/2020

Industry Analysis **Zacks Industry Rank:** Top 28% (72 out of 255)



Top Peers

Ascendis Pharma A/S **(ASND)**	Neutral
Editas Medicine, Inc. **(EDIT)**	Neutral
Senesco Technologies Inc. **(ELOX)**	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 Neutral	RETA Neutral
VGM Score	C	-	-	F	D	F
Market Cap	15.99 B	204.67 M	24.61 B	5.94 B	168.41 M	6.83 B
# of Analysts	10	3	13	6	3	5
Dividend Yield	0.00%	0.00%	1.78%	0.00%	0.00%	0.00%
Value Score	D	-	-	F	F	F
Cash/Price	0.05	0.22	0.04	0.12	0.38	0.04
EV/EBITDA	-5,349.02	-3.70	14.06	-34.29	-2.46	-91.28
PEG Ratio	0.95	1.96	2.09	NA	NA	NA
Price/Book (P/B)	5.20	3.94	3.29	8.08	3.72	NA
Price/Cash Flow (P/CF)	415.78	14.35	13.65	NA	NA	NA
P/E (F1)	50.01	33.65	19.21	NA	NA	NA
Price/Sales (P/S)	9.98	14.39	2.70	245.00	NA	211.50
Earnings Yield	2.00%	-15.58%	5.19%	-3.53%	-33.73%	-3.06%
Debt/Equity	0.27	0.02	0.71	0.05	0.26	-1.11
Cash Flow ($/share)	0.21	-1.07	6.92	-3.63	-1.31	-2.63
Growth Score	A	-	-	F	D	F
Hist. EPS Growth (3-5 yrs)	NA%	16.51%	10.85%	NA	NA	NA
Proj. EPS Growth (F1/F0)	99.49%	7.05%	7.17%	-9.16%	-2.91%	-28.72%
Curr. Cash Flow Growth	-246.03%	19.01%	8.56%	5.18%	129.04%	66.09%
Hist. Cash Flow Growth (3-5 yrs)	21.12%	7.72%	8.36%	NA	NA	NA
Current Ratio	3.77	5.09	1.23	15.74	5.95	3.31
Debt/Capital	21.53%	3.97%	42.91%	4.55%	20.42%	NA
Net Margin	-2.65%	-209.62%	11.81%	-789.80%	NA	-398.84%
Return on Equity	-1.35%	-64.11%	16.86%	-29.04%	-119.84%	-3,281.61%
Sales/Assets	0.36	0.20	0.54	0.03	NA	0.10
Proj. Sales Growth (F1/F0)	15.20%	16.39%	3.85%	-50.47%	NA	45.54%
Momentum Score	F	-	-	A	A	D
Daily Price Chg	0.87%	0.00%	0.06%	1.17%	-0.71%	-1.90%
1 Week Price Chg	3.68%	1.53%	2.47%	6.22%	12.01%	2.56%
4 Week Price Chg	-0.13%	-3.81%	0.59%	0.38%	-10.80%	4.34%
12 Week Price Chg	15.49%	12.80%	6.98%	23.87%	-8.48%	9.64%
52 Week Price Chg	-4.77%	-8.13%	16.62%	99.93%	-64.71%	162.05%
20 Day Average Volume	1,065,504	195,889	2,020,569	121,635	153,877	410,770
(F1) EPS Est 1 week change	0.00%	0.00%	0.00%	0.00%	0.00%	0.00%
(F1) EPS Est 4 week change	-0.23%	0.00%	-0.05%	1.53%	2.63%	0.00%
(F1) EPS Est 12 week change	-0.10%	0.00%	-0.17%	-1.51%	2.63%	-0.07%
(Q1) EPS Est Mthly Chg	-5.88%	0.00%	-0.24%	0.00%	0.00%	0.00%

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	F
VGM Score	C

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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