

# BioMarin Announces First Quarter 2014 Financial Results and VIMIZIM(TM) Launch Progress

**Total BioMarin Revenue Grew 18.5% in 1Q14**

**50 Patients Receiving Commercial VIMIZIM as of April 30**

**VIMIZIM Approved in the U.S. and E.U.; Commercial Launch Underway in both Regions**

**PEG PAL Pivotal Study to be Optimized; Data Expected in 4Q15**

**Financial Highlights (\$ in millions, except per share data, unaudited)**

	<b>Three Months Ended March 31,</b>		
	<b>2014</b>	<b>2013</b>	<b>% Change</b>
Total BioMarin Revenue	\$ 151.5	\$ 127.9	18.5%
Total BioMarin Revenue (excluding Aldurazyme Net Product Transfer Revenue) - non-GAAP	155.3	130.5	19.0%
VIMIZIM Net Product Revenue	0.9	--	--
Naglazyme Net Product Revenue	80.1	69.4	15.4%
Kuvan Net Product Revenue	45.2	37.6	20.2%
Aldurazyme BioMarin Net Product Revenue	18.1	16.7	8.4%
Aldurazyme Royalty Revenue (excluding Net Product Transfer Revenue) - non-GAAP	21.9	19.3	13.5%
Firdapse Net Product Revenue	4.7	3.6	30.6%
Non-GAAP Net Loss	\$ (1.7)	\$ (8.0)	
Non-GAAP Net Loss per Share (basic)	\$ (0.01)	\$ (0.06)	
Non-GAAP Net Loss per Share (diluted)	\$ (0.01)	\$ (0.07)	
GAAP Net Loss	\$ (38.1)	\$ (39.8)	
GAAP Net Loss per Share (basic)	\$ (0.26)	\$ (0.31)	
GAAP Net Loss per Share (diluted)	\$ (0.27)	\$ (0.31)	
Cash, cash equivalents and investments *	\$ 1,138.9	\$ 522.4	

\* The cash balance at the end of March 31, 2014 includes net proceeds of \$696.4 million and \$117.5 million from the Convertible Debt offering in October 2013 and the issuance of common stock in March 2014, respectively.

SAN RAFAEL, Calif., May 1, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced financial results for the first quarter ended March 31, 2014. Non-GAAP net loss was \$1.7 million (\$0.01) per share, basic and diluted for the first quarter of 2014, compared to non-GAAP net loss of \$8.0 million (\$0.06) per share, basic and (\$0.07) per share, diluted for the first quarter of 2013. GAAP net loss was \$38.1 million (\$0.26) per share, basic and (\$0.27) per share diluted for the first quarter of 2014, compared to GAAP net loss of \$39.8 million (\$0.31) per share, basic and diluted for the first quarter of 2013. The decreased non-GAAP net loss and GAAP net loss for the first quarter of 2014 compared to the first quarter of 2013 was primarily due to strong revenue growth across the commercial product portfolio, partially off-set by increased selling, general and administrative expenses, including costs associated with VIMIZIM launch activities, as well as increased research and development expenses.

As of March 31, 2014, BioMarin had cash, cash equivalents and investments totaling \$1,138.9 million, as compared to \$1,052.4 million on December 31, 2013.

"With total BioMarin revenue increasing nearly 20 percent in the quarter and the launch of our next significant growth driver well underway, BioMarin is poised for strong performance in 2014. We are extremely pleased with the momentum of the U.S. commercial launch of VIMIZIM. Since VIMIZIM was approved on February 14<sup>th</sup>, over 120 patients have been referred to the BioMarin Patient and Physician Services (BPPS) channel in the U.S. and 50 patients have started receiving commercial VIMIZIM," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "We now have more patients in the BPPS channel awaiting commercial VIMIZIM than we have on commercial Naglazyme in the U.S., after nine years on the market. We are also very encouraged by the number of patients seeking therapy in Latin America. At the end of the first quarter, there were over 70 patients with prescriptions seeking named patient access in the region. The excitement we are seeing in the Morquio community is driving our expectation of continued, rapid uptake of VIMIZIM across all our commercial markets. Based on these early launch results, we are even more confident in the commercial prospects of VIMIZIM. Having identified over 1,500 MPS IV A patients around the world and with E.U. approval in hand, we believe VIMIZIM sales could help BioMarin reach over \$1 Billion in revenues over the next few years."

### **PEG PAL 165-302 Phase 3 Pivotal Study to be Modified to Increase the Potential of Demonstrating Reduction in Blood Phe and Improvement in Cognitive Function**

The Company has achieved the original enrollment target in the feeder study, BMN165-301 with PEG PAL. Prior to substantially gearing up enrollment in the pivotal randomized Phase 3 trial, BMN 165-302, the Company evaluated Phase 2 data and emerging data from the BMN 165-301 study with PEG PAL. It has previously been established that immunogenicity to PEG PAL, a bacterially derived protein, is responsible for transient, self-limited side effects. In addition, immunogenicity to PEG PAL leads to the need in some patients for higher doses administered more frequently. With the additional data from BMN 165-301 in hand, analyses confirm that immunologic reaction to PEG PAL plays a strong role in determining speed of titration, dose required to lower blood Phe concentrations and safety.

Given this new information, the Company intends to modify the entry criteria for the BMN 165-302 randomized discontinuation study (RDT). The purpose of the eligibility modification is to leverage those patients who have relatively weaker immune reactions to PEG PAL, who therefore achieve Phe reductions faster, at relatively lower doses and with relatively fewer side effects. Under the revised entry criteria, patients will need to demonstrate a pre-specified reduction in blood Phe from PEG PAL during the BMN 165-301 feeder study prior to entering the BMN 165-302 RDT study. By using these criteria, it is expected that approximately 2/3 of patients will meet eligibility criteria for the randomized trial. Therefore, the Company will increase enrollment in the BMN 165-301 feeder study in order to enroll the targeted 120 patients in the BMN 165-302 RDT study. Patients who do not demonstrate a reduction in blood Phe after 24 weeks in the BMN 165-301 study, will enter a parallel study to accommodate the longer time frame

necessary for reaching maximal benefit.

Based on the changed BMN 165-302 protocol, associated review of the proposed protocol and preliminary BMN 165-301 data with the FDA, and requirement for additional patients, the Company now expects data from the enriched BMN 165-302 study to be available in 4Q15.

"We also collected meta analyses that provided evidence that reduction of blood Phe is correlated with improved neurocognitive outcome in PKU patients. Therefore, we continue to be very excited about the potential of PEG PAL to treat adult PKU patients because of its demonstrated ability to lower blood Phe. Our ultimate goal is to demonstrate a significant reduction of blood Phe along with a neurocognitive benefit in all PKU patients, regardless of the time it takes to achieve maximal therapeutic benefit with PEG PAL," stated Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "By requiring a minimum percentage of Phe reduction prior to enrolling in BMN 165-302, we believe we are optimizing the opportunity to demonstrate both Phe lowering and neurocognitive benefits in this pivotal study."

The Company plans to present the new neurocognitive data from the meta analyses at a coming scientific meeting.

## Net Product Revenue

### Total Revenue Growth (in millions)

	Three Months Ended March 31,			
	2014	2013	\$ Change	% Change
VIMIZIM	\$ 0.9	\$ --	\$ 0.9	--
Naglazyme <sup>(1)</sup>	80.1	69.4	10.7	15.4%
Kuvan	45.2	37.6	7.6	20.2%
Aldurazyme	18.1	16.7	1.4	8.4%
Firdapse	4.7	3.6	1.1	30.6%
Net product revenue	149.0	127.3	21.7	17.0%
Collaborative agreement revenue	0.4	0.1	0.3	
Royalty, license and other revenue	2.1	0.5	1.6	
Total BioMarin revenue - GAAP	151.5	127.9	23.6	18.5%
Less: Previously recognized Aldurazyme net product transfer revenue	(3.8)	(2.6)	(1.2)	
Total BioMarin revenues (excluding Aldurazyme net product transfer revenue) - Non-GAAP <sup>(2)</sup>	\$ 155.3	\$ 130.5	\$ 24.8	19.0%

### Reconciliation of Aldurazyme Revenues (in millions)

**Three Months Ended March 31,**

	<b>2014</b>	<b>2013</b>	<b>\$</b>	<b>%</b>
			<b>Change</b>	<b>Change</b>
Aldurazyme revenue reported by Genzyme	\$ 55.9	\$ 48.4	\$ 7.5	15.5%

**Three Months Ended  
March 31,**

	<b>2014</b>	<b>2013</b>	<b>\$</b>
			<b>Change</b>
Aldurazyme Royalties due from Genzyme - Non-GAAP <sup>(2)</sup>	\$ 21.9	\$ 19.3	\$ 2.6
Previously recognized net product transfer revenue <sup>(3)</sup>	(3.8)	(2.6)	(1.2)
Total Aldurazyme net product revenue - GAAP	\$ 18.1	\$ 16.7	\$ 1.4

<sup>(1)</sup> Naglazyme revenues experience quarterly fluctuations due to the timing of government ordering patterns in certain countries. The Company does not believe these fluctuations reflect a change in underlying demand.

<sup>(2)</sup> BioMarin believes that this non-GAAP information is useful to investors, taken in conjunction with BioMarin's GAAP information because it provides additional information regarding the end-user demand for Aldurazyme. The Aldurazyme net product transfer revenue is the result of timing of deliveries to Genzyme Corp. and is therefore not representative of patient demand for the product. By providing information about both the GAAP and non-GAAP revenue measures, the Company believes that the additional information enhances investors' overall understanding of the Company's business and in particular allows for more consistent period to period evaluation of the revenue.

<sup>(3)</sup> To the extent units shipped to third party customers by Genzyme exceed BioMarin inventory transfers to Genzyme, BioMarin will record a decrease in net product revenue from the royalty payable to BioMarin for the amount of previously recognized product transfer revenue. If BioMarin inventory transfers exceed units shipped to third party customers by Genzyme, BioMarin will record incremental net product transfer revenue for the period.

**No Change to 2014 Guidance**

Revenue Guidance (\$ in millions)

<u>Item</u>	<u>2014 Guidance</u>	
Total BioMarin Revenues	\$650 to \$680	Trending toward the upper-end of guidance
Naglazyme Net Product Revenue	\$290 to \$310	
Kuvan Net Product Revenue	\$180 to \$200	
VIMIZIM	\$60 to \$70	
Selected Income Statement Guidance (\$ in millions)		

<u>Item</u>	<u>2014 Guidance</u>
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Cost of Sales (% of Total Revenue)	17.5% to 18.5%	
Selling, General and Admin. Expense	\$265 to \$285	
Research and Development Expense	\$500 to \$530	Trending toward the lower-end of guidance
GAAP Net Loss	\$(255) to \$(285)	Trending toward the lower-end of guidance
Non-GAAP Net Loss	\$(100) to \$(130)	Trending toward the lower-end of guidance

### **Anticipated Upcoming Milestones**

2Q 2014: Initiation of Phase 2/3 trial with BMN 701 for the treatment of Pompe disease

2Q 2014: EU launch of VIMIZIM for MPS IVA

4Q 2014: Enrollment completion of Phase 2 trial with BMN 111 for the treatment of achondroplasia

4Q 2014: Enrollment completion of Phase 1/2 trial with BMN 190 for the treatment of Batten disease

1Q 2015: IND filing or equivalent for BMN 270 for the treatment of Hemophilia A

2Q 2015: Data on first three cohorts in Phase 1/2 with BMN 111 for the treatment of achondroplasia

1H 2015: Enrollment completion of Phase 2/3 trial with BMN 701 for the treatment of Pompe disease

2H 2015: Results from Phase 1/2 trial with BMN 190 for the treatment of Batten disease

2H 2015: Enrollment completion of Phase 3 trial with BMN 673 for the treatment of mBC

Mid-2015: IND filing or equivalent for BMN 250 for the treatment of MPS IIIB

4Q 2015: Results from pivotal Phase 3 trial with PEG PAL for the treatment of PKU

1Q 2016: Submission of PEG PAL BLA to the FDA for the treatment of PKU

### **Commercial and Regulatory Update on VIMIZIM for Mucopolysaccharidosis type IVA**

- U.S.:** On February 14, 2014 the U.S. Food and Drug Administration (FDA) approved VIMIZIM (elosulfase alfa) for the treatment of all patients with Mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome) regardless of age or disease progression. Following approval, shipments of VIMIZIM to appropriate distribution channels and promotion in the U.S. began immediately. U.S. early access patients and clinical trial patients are being transitioned to commercial VIMIZIM. As of the end of April, over 120 patients were in the BioMarin Patient and Physician Services (BPPS) channel in the U.S. and 50 patients were receiving commercial VIMIZIM.
- E.U.:** On April 28, 2014 the European Commission granted marketing authorization for VIMIZIM, the first specific treatment approved in the European Union for Mucopolysaccharidosis type IVA in patients of all ages. As the first drug ever approved for Morquio A syndrome, VIMIZIM has been granted orphan drug status in the European Union, which confers ten years of market exclusivity. The commercial launch of VIMIZIM in the E.U. is currently underway.

### **Advanced Clinical Programs**

- **Phase 3 with BMN 673 (PARP inhibitor) for gBRCA breast cancer:** The Company is conducting a Phase 3 trial to study its poly ADP-ribose polymerase (PARP) inhibitor, BMN 673, for the treatment of deleterious germline BRCA mutation metastatic breast cancer and is currently enrolling patients. The Phase 3 trial is an open-label, 2:1 randomized, parallel, two-arm study of BMN 673 as compared to monotherapy of physicians' choice (capecitabine, eribulin, gemcitabine or vinorelbine) in germline BRCA mutation subjects with locally advanced and/or metastatic breast cancer who have received no more than two prior chemotherapy regimens for metastatic disease. The global study will enroll approximately 429 subjects. The primary objective of the study is to compare progression-free survival (PFS) of subjects treated with BMN 673 as a monotherapy relative to those treated with protocol-specific physicians' choice. The Company plans to complete enrollment in 2H15.
- **Phase 2/3 with BMN 701 for Pompe Disease:** A phase 2/3 trial of patients previously treated with alglucosidase alfa and switched to a treatment of BMN 701 at 20 mg/kg administered every other week for 24 weeks is being prepared and patients are currently being screened to enroll in the study. The primary endpoint of the study will be the respiratory parameter Maximal Inspiratory Pressure (MIP). The Company plans to complete enrollment in 1H15.
- **Phase 2 with BMN 111 for Achondroplasia:** In January 2014, the Company announced that it had dosed the first child in the Phase 2 trial with BMN 111, an analog of C-type Natriuretic Peptide (CNP), for the treatment of children with achondroplasia. Achondroplasia is the most common form of disproportionate short stature or dwarfism. The Phase 2 study is an open-label, sequential cohort, dose-escalation study of BMN 111 in children who are 5-14 years old. The primary objective of this study is to assess the safety and tolerability of daily subcutaneous doses of BMN 111 administered for 6 months. Prior to enrolling in the Phase 2 study, all patients will have participated in a 6 month natural history study to determine baseline growth velocity data. The Company expects results from the first three cohorts in this study in 2Q15.

### Early-Stage Clinical Programs

- **BMN 190 for LINCL (Batten disease):** The Company is conducting a Phase 1/2 trial with BMN 190, a recombinant human tripeptidyl peptidase 1 (rhTPP1) for the treatment of patients with neuronal ceroid lipofuscinosis type 2 (NCL-2), a form of Batten disease. This is the first time that patients with Batten Disease have been treated with an enzyme replacement therapy in a clinical trial setting. The Phase 1/2 study is an open-label, dose-escalation study in patients with NCL-2. The primary objectives are to evaluate the safety and tolerability of BMN 190 and to evaluate effectiveness using an NCL-2-specific rating scale score in comparison with natural history data after 48 weeks of treatment. Secondary objectives are to evaluate the impact of treatment on brain atrophy in comparison with NCL-2 natural history after 48 weeks of treatment and to characterize pharmacokinetics and immunogenicity. The study will enroll approximately 22 subjects for a treatment duration of 48 weeks. The Company expects enrollment completion in 4Q14 and results from this study in 2H15.

### Preclinical Programs

- **BMN 270 for Hemophilia A:** In January 2014, the Company announced that it had selected an AAV-factor VIII gene therapy drug candidate, BMN 270, to develop for the treatment of hemophilia A and has initiated IND-enabling studies. BioMarin expects to initiate clinical studies with BMN 270 in early 2015.
- **BMN 250 for MPS IIIB:** In February 2014, the Company announced that it had selected an BMN 250, an intracerebroventricular enzyme replacement therapy, for the treatment of Mucopolysaccharidosis IIIB (MPS IIIB) or Sanfilippo Syndrome Type B (Sanfilippo B). BioMarin has initiated IND-enabling

studies and expects to initiate clinical studies with BMN 250 in Mid-2015.

## Non-GAAP Net Loss and Reconciliation

The results for the three months ended March 31, 2014 and March 31, 2013 and the guidance for 2014 include both GAAP net loss and non-GAAP net loss. As used in this release, non-GAAP net loss is based on GAAP earnings before interest, taxes, depreciation and amortization (EBITDA) and further adjusted to exclude certain non-cash stock compensation expense, non-cash contingent consideration expense and certain other nonrecurring material items (non-GAAP net loss).

The following table presents the reconciliation of GAAP to non-GAAP financial metrics:

### Reconciliation of GAAP Net Loss to Non-GAAP Net Loss

(in millions)

(unaudited)

	Three Months Ended March 31,		Year Ending December 31, 2014
	2014	2013	Guidance
<b>GAAP Net Loss</b>	<b>\$ (38.1)</b>	<b>\$ (39.8)</b>	<b>\$(255.0) - \$(285.0)</b>
Interest expense, net	8.0	1.0	35.0
Provision for (benefit from) income taxes	3.5	(4.7)	(15.0)
Depreciation expense	6.7	6.1	35.0
Amortization expense	2.6	2.6	10.5
<b>EBITDA Loss</b>	<b>(17.3)</b>	<b>(34.8)</b>	<b>(189.5) - (219.5)</b>
Stock-based compensation expense	16.3	11.6	80.5
Contingent consideration expense <sup>(1)</sup>	8.1	4.8	17.8
Material non-recurring:			
Gain on termination of lease <sup>(2)</sup>	(8.8)	--	(8.8)
Debt conversion expense <sup>(3)</sup>	--	10.4	--
<b>Non-GAAP Net Loss</b>	<b>\$ (1.7)</b>	<b>\$ (8.0)</b>	<b>\$(100.0) - \$(130.0)</b>

<sup>(1)</sup> Represents the expense associated with the change in the fair value of contingent acquisition consideration payable for the period, resulting from changes in estimated probabilities and timing of achieving certain developmental milestones.

<sup>(2)</sup> Represents the net gain due to the early termination of the Company's operating lease and the realization of the remaining balance in deferred rent upon acquisition of the San Rafael Corporate Center where the Company's corporate headquarters are located.

(3) Represents debt conversion expense associated with the early conversion of a portion of our 2017 convertible notes.

BioMarin believes that this non-GAAP information is useful to investors, taken in conjunction with BioMarin's GAAP information because it provides additional information regarding the performance of BioMarin's core ongoing business, VIMIZIM, Naglazyme, Kuvan, Aldurazyme and Firdapse, and development of the Company's pipeline. By providing information about both the overall GAAP financial performance and the non-GAAP measures that focus on continuing operations, the Company believes that the additional information enhances investors' overall understanding of the Company's business and prospects for the future. Further, the Company uses both the GAAP and the non-GAAP results and expectations internally for its operating, budgeting and financial planning purposes.

### **Conference Call Details**

BioMarin will host a conference call and webcast to discuss first quarter 2014 financial results today, Thursday, May 1, at 5:00 p.m. ET. This event can be accessed on the investor section of the BioMarin website at [www.BMRN.com](http://www.BMRN.com).

U.S. / Canada Dial-in Number: 877.303.6313  
International Dial-in Number: 631.813.4734  
Conference ID: 30354748

Replay Dial-in Number: 855.859.2056  
Replay International Dial-in Number: 404.537.3406  
Conference ID: 30354748

### **About BioMarin**

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises five approved products and multiple clinical and pre-clinical product candidates. Approved products include VIMIZIM™ (elosulfase alfa) for MPS IVA; Naglazyme® (galsulfase) for MPS VI; Aldurazyme® (laronidase) for MPS I, a product which BioMarin developed through a 50/50 joint venture with Genzyme, a Sanofi Company; Kuvan® (sapropterin dihydrochloride) Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany and Firdapse™ (amifampridine), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Product candidates include PEG PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase 3 clinical development for the treatment of PKU, BMN 673, a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase 3 clinical development for the treatment of germline BRCA breast cancer, BMN 701, a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA), which is currently in Phase 1/2 clinical development for the treatment of Pompe disease, BMN 111, a modified C-natriuretic peptide, which is currently in Phase 1 clinical development for the treatment of achondroplasia, BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1) for the treatment of late-infantile neuronal ceroid lipofuscinosis (CLN2), a form of Batten Disease, which is currently in Phase 1, BMN 270, an AAV-factor VIII vector, for the treatment of hemophilia A and BMN 250, a novel fusion of alpha-N-acetylglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of MPS IIIB. For additional information, please visit [www.BMRN.com](http://www.BMRN.com).

### **Forward-Looking Statement**

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: the expectations of revenue and sales related to VIMIZIM, Naglazyme, Kuvan, Aldurazyme and Firdapse; the financial performance of the BioMarin as a whole; the timing of BioMarin's clinical trials of PEG PAL, BMN 673, BMN 701, BMN 111, BMN 190, BMN 270, BMN 250 and other product candidates; the continued clinical development and commercialization of Aldurazyme, Naglazyme, Kuvan, Firdapse, VIMIZIM and its product candidates; and actions by regulatory authorities. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others: our success in the commercialization of VIMIZIM, Naglazyme, Kuvan, and Firdapse; Genzyme Corporation's success in continuing the commercialization of Aldurazyme; results and timing of current and planned preclinical studies and clinical trials, particularly with respect to PEG PAL, BMN 673, BMN 701, BMN 111 and BMN 190; our ability to successfully manufacture our products and product candidates; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities concerning each of the described products and product candidates; the market for each of these products and particularly Aldurazyme, Naglazyme, Kuvan, VIMIZIM and Firdapse; actual sales of Aldurazyme, Naglazyme, Kuvan, VIMIZIM and Firdapse; Merck Serono's activities related to Kuvan; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission, including, without limitation, the risk factors contained under the caption "Risk Factors" in BioMarin's 2013 Annual Report on Form 10-K, and the factors contained in BioMarin's reports on Form 10-Q. Stockholders are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin is under no obligation, and expressly disclaims any obligation to update or alter any forward-looking statement, whether as a result of new information, future events or otherwise.

BioMarin<sup>®</sup>, Naglazyme<sup>®</sup> and Kuvan<sup>®</sup> are registered trademarks of BioMarin Pharmaceutical Inc., or its affiliates. Aldurazyme<sup>®</sup> is a registered trademark of BioMarin/Genzyme LLC. VIMIZIM<sup>™</sup> and Firdapse<sup>™</sup> are trademarks of BioMarin Pharmaceutical Inc., or its affiliates.

## **BIOMARIN PHARMACEUTICAL INC.**

### **CONDENSED CONSOLIDATED BALANCE SHEETS**

**March 31, 2014 and December 31, 2013**

**(In thousands of U.S. dollars, except share and per share amounts)**

	<b>March 31,</b>	<b>December</b>
	<b>2014</b>	<b>31,</b>
		<b>2013(1)</b>
	(unaudited)	
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 639,778	\$ 568,781
Short-term investments	247,703	215,942
Accounts receivable, net (allowance for doubtful accounts: \$551 and \$529, respectively)	110,462	117,822

Inventory	176,893	162,605
Current deferred tax assets	30,561	30,561
Other current assets	41,013	41,707
Total current assets	1,246,410	1,137,418
Noncurrent assets:		
Investment in BioMarin/Genzyme LLC	478	816
Long-term investments	251,450	267,700
Property, plant and equipment, net	437,066	319,316
Intangible assets, net	165,397	163,147
Goodwill	54,258	54,258
Long-term deferred tax assets	144,124	145,234
Other assets	41,545	156,171
Total assets	\$ 2,340,728	\$ 2,244,060
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$ 156,038	\$ 183,271
Total current liabilities	156,038	183,271
Noncurrent liabilities:		
Long-term convertible debt	661,419	655,566
Long-term contingent acquisition consideration payable	38,430	30,790
Other long-term liabilities	24,720	33,392
Total liabilities	880,607	903,019
Stockholders' equity:		
Common stock, \$0.001 par value: 250,000,000 shares authorized at March 31, 2014 and December 31, 2013; 145,738,396 and 143,463,668 shares issued and outstanding at March 31, 2014 and December 31, 2013, respectively.	146	144
Additional paid-in capital	2,213,347	2,059,101
Company common stock held by Nonqualified Deferred Compensation Plan	(6,731)	(7,421)
Accumulated other comprehensive income	7,275	5,018
Accumulated deficit	(753,916)	(715,801)
Total stockholders' equity	1,460,121	1,341,041
Total liabilities and stockholders' equity	\$ 2,340,728	\$ 2,244,060

(1) December 31, 2013 balances were derived from the audited consolidated financial statements.

**BIOMARIN PHARMACEUTICAL INC.**

**CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS****Three Months Ended March 31, 2014 and 2013****(In thousands of U.S. dollars, except per share amounts)****(Unaudited)**

	<b>Three Months Ended March 31,</b>	
	<b>2014</b>	<b>2013</b>
<b>REVENUES:</b>		
Net product revenues	\$ 149,004	\$ 127,344
Collaborative agreement revenues	415	135
Royalty, license and other revenues	2,133	449
Total revenues	151,552	127,928
<b>OPERATING EXPENSES:</b>		
Cost of sales (excludes amortization of certain acquired intangible assets)	22,816	20,500
Research and development	86,166	83,743
Selling, general and administrative	60,069	51,050
Intangible asset amortization and contingent consideration	8,957	5,556
Total operating expenses	178,008	160,849
<b>LOSS FROM OPERATIONS</b>	<b>(26,456)</b>	<b>(32,921)</b>
Equity in the loss of BioMarin/Genzyme LLC	(338)	(401)
Interest income	1,123	718
Interest expense	(9,106)	(1,725)
Debt conversion expense	--	(10,420)
Other income	153	228
<b>LOSS BEFORE INCOME TAXES</b>	<b>(34,624)</b>	<b>(44,521)</b>
Provision for (benefit from) income taxes	3,491	(4,711)
<b>NET LOSS</b>	<b>\$ (38,115)</b>	<b>\$ (39,810)</b>
<b>NET LOSS PER SHARE, BASIC</b>	<b>\$ (0.26)</b>	<b>\$ (0.31)</b>
<b>NET LOSS PER SHARE, DILUTED</b>	<b>\$ (0.27)</b>	<b>\$ (0.31)</b>
Weighted average common shares outstanding, basic	143,983	127,969
Weighted average common shares outstanding, diluted	144,157	127,969
<b>COMPREHENSIVE LOSS</b>	<b>\$ (35,858)</b>	<b>\$ (38,453)</b>

**STOCK-BASED COMPENSATION EXPENSE**

Total stock-based compensation expense included in the Condensed Consolidated Statements of Comprehensive Loss is as follows (unaudited):

	<b>Three Months Ended March 31,</b>	
	<b>2014</b>	<b>2013</b>
Cost of sales	\$ 1,086	\$ 1,044
Research and development	7,115	5,324
Selling, general and administrative	8,103	5,197
	<b>\$ 16,304</b>	<b>\$ 11,565</b>

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<http://investors.biomarin.com/2014-05-01-BioMarin-Announces-First-Quarter-2014-Financial-Results-and-VIMIZIM-TM-Launch-Progress>