

ORPHAN MEDICAL INC  
Form 10-Q  
August 09, 2004

**SECURITIES AND EXCHANGE COMMISSION**

**WASHINGTON, D.C. 20549**

**FORM 10-Q**

(Mark One)

[X]

Quarterly Report pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934 for the quarterly period ended June 30, 2004

[ ]

Transition report pursuant to section 13 or 15(d) of the Securities Exchange Act of 1934 for the transition

period from \_\_\_\_\_ to \_\_\_\_\_

Commission File Number

0-24760

**Orphan Medical, Inc.**

(Exact name of registrant as specified in its charter)

**Delaware**

(State or other jurisdiction of

**41-1784594**

(I.R.S. Employer Identification

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incorporation or organization)  
  
**13911 Ridgedale Drive, Suite 250,  
Minnetonka, MN 55305**  
(Address of principal executive office  
and zip code)

Number)  
  
**(952) 513-6900**  
(Registrant's telephone number,  
including area code)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months, and (2) has been subject to such filing requirements for the past 90 days.

Yes  No

Indicate by check mark whether the registrant is an accelerated filer (as defined in Rule 12b-2 of the Exchange Act).

Yes  No

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practical date.

Common Stock, \$.01 par value

11,278,671

(Class)

(Outstanding at August 1, 2004)

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Antizol®, Antizol-Vet®, Cystadane®, Xyrem®, MedExpand® The Orphan Drug Company Orphan Medical and Dedicated to Patients with Uncommon Diseases® are trademarks of the Company.

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PART I FINANCIAL INFORMATION

**Item 1.** Financial Statements

**ORPHAN MEDICAL, INC.  
BALANCE SHEETS  
(in thousands except share and per share amounts)**

	<b>June 30, 2004</b>	<b>December 31, 2003</b>
	<hr/>	
	(Unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	\$ 15,261	\$ 23,285
Restricted cash	128	128
Accounts receivable, less allowance for doubtful accounts of \$100 and \$112, respectively	2,481	2,552
Inventories	1,888	1,696

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	June 30, 2004	December 31, 2003
Prepaid expenses and other	807	907
Total current assets	20,565	28,568
Office equipment and software	675	754
Total assets	\$ 21,240	\$ 29,322
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable	\$ 1,654	\$ 2,923
Accrued compensation	1,115	881
Deferred revenue	2,083	2,500
Accrued expenses and other	3,772	2,460
Total current liabilities	8,624	8,764
Capital lease obligation-less current maturities	53	62
Commitments		
Shareholders' equity:		
Senior Convertible Preferred Stock, \$.01 par value; 14,000 shares authorized; 8,706 shares issued and outstanding; liquidation preference of \$8,706		
Series B Convertible Preferred Stock, \$.01 par value; 5,000 shares authorized; 4,106 and 3,957 shares issued and outstanding; liquidity preference of \$4,106 and \$3,957		
Series C Convertible Preferred Stock, \$.01 par value; 4,000 shares authorized; 0 shares issued and outstanding		
Series D Convertible Preferred Stock, \$.01 par value; 1,000 shares authorized; 0 shares issued and outstanding		
Common stock, \$.01 par value; 25,000,000 shares authorized; 10,900,549 and 10,747,656 issued and outstanding	109	107
Additional paid-in capital	77,945	76,714
Accumulated deficit	(65,491)	(56,325)
Total shareholders' equity	12,563	20,496
Total liabilities and shareholders' equity	\$ 21,240	\$ 29,322

**Note:** The Balance Sheet at December 31, 2003 has been derived from the audited financial statements at that date but does not include all of the information and footnotes required by generally accepted accounting principles for complete financial statements.

See accompanying notes.

**Orphan Medical, Inc.**  
**Statements of Operations**  
(in thousands except for per share amounts)  
(Unaudited)

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2004	2003	2004	2003
Product revenue, net	\$ 4,995	\$ 4,349	\$ 9,398	\$ 8,916
Licensing and royalty revenue	417		1,417	
<b>Total revenue</b>	<b>5,412</b>	<b>4,349</b>	<b>10,815</b>	<b>8,916</b>
Operating expenses:				
Cost of sales	734	717	1,365	1,464
Product development	2,965	2,320	7,187	4,486
Sales and marketing	5,366	3,705	8,764	7,958
General and administrative	1,049	1,384	2,256	2,643
<b>Total operating expenses</b>	<b>10,114</b>	<b>8,126</b>	<b>19,572</b>	<b>16,551</b>
Loss from operations	(4,702)	(3,777)	(8,757)	(7,635)
Interest income	40	14	91	36
Interest expense	(1)	(35)	(22)	(49)
Gain on divestment of products		30,267		30,267
<b>Net income (loss) before taxes</b>	<b>(4,663)</b>	<b>26,469</b>	<b>(8,688)</b>	<b>22,619</b>
Income tax expense		250		257
<b>Net income (loss)</b>	<b>(4,663)</b>	<b>26,219</b>	<b>(8,688)</b>	<b>22,362</b>
Less: Preferred stock dividends	240	234	478	466
<b>Net income (loss) attributable to common shareholders</b>	<b>\$ (4,903)</b>	<b>\$ 25,985</b>	<b>\$ (9,166)</b>	<b>\$ 21,896</b>
<b>Earnings (loss) per share</b>				
Basic	\$ (0.45)	\$ 2.47	\$ (0.85)	\$ 2.08
Diluted	\$ (0.45)	\$ 2.06	\$ (0.85)	\$ 1.75
<b>Weighted average number of shares used to calculate earnings (loss) per share</b>				
Basic	10,852	10,538	10,814	10,517
Diluted	10,852	12,709	10,814	12,781

See accompanying notes.

**Orphan Medical, Inc.**  
**Statements of Cash Flows**  
(in thousands)  
(Unaudited)

	For the Six Months Ended June 30	
	2004	2003
<b>Operating activities</b>		
Net (loss) income	\$ (8,688)	\$ 22,362
Adjustments to reconcile net income to net cash used in operating activities:		
Depreciation and amortization	243	247
Gain on disposition of products		(30,267)
Changes in operating assets and liabilities:		
Accounts receivable and current assets	171	(197)
Inventories	(192)	699
Accounts payable, accrued expenses and deferred revenue	(140)	(914)
<b>Net cash used in operating activities</b>	<b>(8,606)</b>	<b>(8,070)</b>
<b>Investing activities</b>		
Purchase of office equipment	(145)	(34)
Net proceeds from disposition of products		30,267
<b>Net cash provided by (used in) investing activities</b>	<b>(145)</b>	<b>30,233</b>
<b>Financing activities:</b>		
Employee stock purchase plan	29	23
Stock option exercise proceeds	705	622
Private common stock placement		
Principal payments on capital lease	(7)	(8)
Cash dividends		(1)
<b>Net cash provided by financing activities</b>	<b>727</b>	<b>636</b>
Increase (decrease) in cash and cash equivalents	(8,024)	22,798
Cash and cash equivalents at beginning of period	23,285	6,921
<b>Cash and cash equivalents at end of period</b>	<b>\$ 15,261</b>	<b>\$ 29,720</b>

*See accompanying notes.*

## ORPHAN MEDICAL, INC.

NOTES TO FINANCIAL STATEMENTS  
(Unaudited)**1. Basis of Presentation*****Business***

Orphan Medical acquires, develops, and markets products of high medical value intended to treat sleep disorders, pain and other central nervous system disorders that are addressed by physician specialists. A drug has high medical value if it offers a major improvement in the safety or efficacy of patient treatment and has no substantially equivalent substitute. The Company has had six pharmaceutical products approved for marketing by the United States Food and Drug Administration (FDA). Three were divested in 2003 and the Company is focusing its resources on Xyrem® (sodium oxybate) oral solution, a medication approved for cataplexy, a significant and debilitating symptom of narcolepsy. The Company is conducting clinical trials to assess Xyrem in treating excessive daytime sleepiness and fragmented nighttime sleep, the other prominent symptoms of narcolepsy. The Company is also evaluating Xyrem in the treatment of fibromyalgia. A new compound, Butamben (butyl-p-aminobenzoate) suspension for injection, is being evaluated for development as a treatment of pain. The Company is seeking to acquire other approved or development-stage products in the specialty areas it serves.

***Basis of Presentation***

The accompanying unaudited financial statements have been prepared in accordance with generally accepted accounting principles for interim financial information and with the instructions to Form 10-Q and Article 10 of Regulation S-X. Accordingly, these financial statements do not include all of the information and footnotes required by generally accepted accounting principles for complete financial statements. In the opinion of management, all adjustments (consisting of normal, recurring accruals) considered necessary for fair presentation have been included. Operating results for the three-month and six-month period ended June 30, 2004 are not necessarily indicative of the results that may be expected for the year ended December 31, 2004. For further information, refer to the audited financial statements and accompanying notes contained in the Company's amended Annual Report filed on Form 10-K for the year ended December 31, 2003.

**2. Disposition of products**

On June 10, 2003, the Company announced the disposition of Busulfex® (busulfan) Injection to ESP Pharma, Inc. for \$29.3 million plus the book value of inventory, approximately \$0.2 million. The Company announced the sale of the product Sucraid® (sacrosidase) oral solution to a specialty pharmaceutical company on May 6, 2003 for \$1.5 million. The Company also divested a third product, Elliotts B Solution® to another company for proceeds that were not material. Proceeds from these dispositions are being used for further development and marketing of Xyrem® (sodium oxybate) oral solution and for building a stronger presence in the sleep and central nervous system (CNS) markets.

**3. Use of Estimates**

The preparation of financial statements in conformity with accounting principles generally accepted in the United States requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Actual results could differ from those estimates.

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**4. Stock-Based Compensation**

At June 30, 2004 the Company has a stock-based employee compensation plan. The Company accounts for its plan under the recognition and measurement principles of Accounting Principles Board Opinion No. 25, *Accounting for Stock Issued to Employees*, and related interpretations. No stock-based compensation cost is reflected in the net loss for the three or six month periods ended June 30, 2004 or 2003, as all options granted under this plan had an exercise price equal to market value of the underlying common stock on the date of grant.

The following table illustrates the effect on net loss and net loss per share if the Company had applied the fair value recognition provisions of Statement of Financial Accounting Standards No. 123, *Accounting for Stock-Based Compensation*, to stock-based employee compensation.

(in thousands except per share data)	Three months ended		Six months ended	
	June 30,		June 30,	
	2004	2003	2004	2003

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(in thousands except per share data)	Three months ended		Six months ended	
	June 30,		June 30,	
Net (loss) income as reported	\$ (4,903)	\$ 25,985	\$ (9,166)	\$ 21,896
Deduct total stock-based employee compensation expense determined under fair value-based method for all awards	(400)	(683)	(972)	(1,257)
Pro forma net income (loss)	\$ (5,303)	\$ 25,302	\$ (10,138)	\$ 20,638
Earnings (loss) per share				
Basic as reported	\$ (0.45)	\$ 2.47	\$ (0.85)	\$ 2.08
Basic as pro forma	\$ (0.49)	\$ 2.40	\$ (0.94)	\$ 1.96
Diluted as reported	\$ (0.45)	\$ 2.06	\$ (0.85)	\$ 1.75
Diluted as pro forma	\$ (0.49)	\$ 2.16	\$ (0.94)	\$ 1.61

## 5. Revenue Recognition

Sales for all products, except Xyrem, are recognized at the time a product is shipped to the Company's customers and are recorded net of reserves for discounts for prompt payment. Sales of Xyrem are recognized at the time product is shipped from the specialty pharmacy to the patient and are recorded net of discounts for prompt payment. Except for Xyrem, the Company is obligated to accept, for exchange, from all domestic customers products that have reached their expiration date, which range from three to five years depending on the product. The Company is not obligated to accept exchange of outdated product from its international distribution partners. The Company establishes a reserve for the estimated cost of the exchanges. The Company monitors the exchange of product and modifies its reserve as necessary. Management bases these reserves on historical experience and these estimates are subject to change.

Deferred revenue represents the initial payment received by the Company per the terms of the Company's license agreement for Xyrem with Celltech Pharmaceuticals, a division of Celltech Group plc (Celltech). Upon expiration of the refund conditions on April 1, 2004, this fee is being recognized over the expected regulatory review period. The Company will recognize this fee over the estimated 18 month review period. The Company recognized \$0.4 million of income resulting from the amortization of the deferred revenue balance.

The Company received \$1.0 million during the quarter ended March 31, 2004 as payment for the achievement of a milestone in the license agreement. This payment is included in Licensing and royalty revenue in the Company's Statement of Operations. Future milestone payments are expected to be recognized as earned.

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## 6. Inventories

Inventories are valued at the lower of cost or market determined using the first-in, first-out (FIFO) method. The Company's policy is to establish an excess and obsolete reserve for its products in excess of the expected demand for such products.

	June 30, 2004	December 31, 2003
Raw materials and packaging	\$ 722	\$ 690
Finished goods	1,166	1,006
	\$ 1,888	\$ 1,696



**7. Earnings per Share**

Earnings per share are computed in accordance with SFAS No. 128, Earnings per Share. Basic earnings (loss) per share are computed based on the weighted average number of common shares outstanding during the period. Diluted earnings (loss) per share are computed based on the weighted average shares outstanding and the dilutive impact of common stock equivalents outstanding during the period. The dilutive effect of employee stock options and warrants is measured using the treasury stock method. The dilutive effect of both series of convertible preferred stock is computed using the if-converted method. Common stock equivalents are not included in periods where there is a loss, as they are antidilutive. The following is a reconciliation of net income (loss) and weighted average common shares outstanding for purposes of calculating basic and diluted earnings (loss) per share:

	(in thousands, except pershare data)			
	Three months ended June 30,		Six months ended June 30,	
	2004	2003	2004	2003
<i>Numerator</i>				
Numerator for basic earnings per share				
income available to common shareholders	\$ (4,903)	\$ 25,985	\$ (9,166)	\$ 21,896
Add back to effect assumed conversions:				
Preferred stock dividends		234		466
Numerator for diluted earnings per share	\$ (4,903)	\$ 26,219	\$ (9,166)	\$ 22,362
<i>Denominator</i>				
Denominator for basic earnings per share				
weighted average shares	10,852	10,538	10,814	10,517
Effect of dilutive securities:				
Preferred shares		1,657		1,653
Stock options		312		380
Warrants		202		231
Denominator for diluted earnings per share				
weighted average shares and assumed conversions	10,852	12,709	10,814	12,781
Basic earnings per share	\$ (0.45)	\$ 2.47	\$ (0.85)	\$ 2.08
Diluted earnings per share	\$ (0.45)	\$ 2.06	\$ (0.85)	\$ 1.75

**8. Commitments**

The Company has various commitments under agreements with outside consultants and contractors to provide services relating to drug development, drug acquisition, manufacturing and marketing. At June 30, 2004, the Company estimates that it could incur approximately \$7.1 million of additional expenditures in subsequent periods under existing commitments. Commitments for research and development expenditures will likely fluctuate from quarter to quarter and from year to year depending on, among other factors, the timing of product development and the progress of clinical development programs.

**9. Borrowings**

The Company has a line of credit facility with a commercial bank expiring September 30, 2004. The line of credit facility has a term of one-year and includes a borrowing base equal to 75% of eligible accounts receivable up to a maximum amount of \$2.5 million. Certain other assets have

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also been pledged as collateral for this facility. The interest rate is equal to two points over the bank's prime rate. The Company is also subject to certain other requirements during the term of the facility, including (a) minimum quarterly net tangible equity of \$5.0 million plus 50 percent of the proceeds of any equity securities or subordinated debt offering and (b) maximum monthly operating loss of \$2.7 million for April, May and June 2004 and \$2.0 million for July, August and September 2004. The Company was in compliance with these covenants as of June 30, 2004. The Company had not borrowed under this facility at June 30, 2004. The Company's borrowing base as of June 30, 2004 was approximately \$1.7 million.

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### Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

#### Cautionary Statement

This Quarterly Report on Form 10-Q contains statements that are not descriptions of historical facts. The words or phrases "will likely result", "look for", "may result", "will continue", "is anticipated", "expect", "project", or similar expressions are intended to identify forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Such statements may be forward-looking statements that are subject to risks and uncertainties. Actual results could differ materially from those currently anticipated due to a number of factors, including those identified in the section of this Quarterly Report filed on Form 10-Q for the quarterly period ended June 30, 2004 titled Risk Factors.

#### General

Since its inception, the activities of the Company have consisted primarily of obtaining the rights to proposed pharmaceutical products for developing and marketing, managing the development of these products and preparing for and initiating the commercial introduction of six products. The Company operates in a single business segment: pharmaceutical products. The Company has experienced recurring losses from operations and has generated an accumulated deficit through June 30, 2004 of \$65.5 million. In addition, the Company expects to incur additional losses from operations for at least for the next several fiscal quarters.

#### Three Months Ended June 30, 2004 vs. Three Months Ended June 30, 2003

Net loss applicable to common shareholders was \$4.9 million for the three months ended June 30, 2004 compared to a net income applicable to common shareholders of \$26.0 million for the three months ended June 30, 2003. This change is attributable to the product dispositions during the quarter ended June 30, 2003. These product dispositions resulted in a net gain of \$30.3 million. Operating expenses increased over the prior year primarily due to the commercialization of Xyrem and the ongoing development activities for Xyrem.

#### Product Revenue Summary

The following is a summary of product revenue for the three months ended June 30, 2004 compared to the three months ended June 30, 2003:

000s	Three Months ended June 30,		Variance	
	2004	2003	\$	%
Antizol	\$ 2,274	\$ 1,759	\$ 515	29%
Antizol-Vet	66	44	22	50
Cystadane	417	271	146	54
Xyrem	2,238	775	1,463	188

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	Three Months ended June 30,		Variance	
Busulfex (1)	1,468		(1,468)	(100)
Elliotts B (1)	(4)		4	(100)
Sucraid (1)	35		(35)	(100)
<b>Total</b>	\$ 4,995	\$ 4,349	\$ 646	15%

(1) These products were divested during the second quarter of 2003.

Net sales increased 15% to \$5.0 million for the three months ended June 30, 2004 compared to \$4.3 million for the same period in the prior year. Sales of Xyrem were approximately \$800,000 for the quarter ended June 30, 2003 and \$2.2 million for the quarter ended June 30, 2004. Revenue from products marketed by the Company during the three months ended June 30, 2004 increased 75% compared to the same products in the corresponding 2003 period. Net sales for the quarter ended June 30, 2003 include approximately \$1.5 million from divested products prior to the divestiture of these products.

During the second half of the quarter the Company experienced a large increase in the number of new prescriptions written for Xyrem. The Company's market research indicates that overall satisfaction by both patients and physicians continues to be strong and third party reimbursement remained high.

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Sales of Antizol® (fomepizole) Injection increased 29% compared to the prior year as more hospitals with emergency rooms stocked the product and the market share of Antizol continued to increase for the treatment of ethylene glycol or methanol poisoning.

Due to product mix, gross profit margins increased to 85% for the quarter ended June 30, 2004 compared to 84% for the same period the prior year. Cost of sales was \$0.7 million for the three months ended June 30, 2004 equivalent to the same period the prior year. The three products divested in the second quarter of 2003 had slightly lower margins than the Company's current product portfolio. Cost of sales as a percentage of net sales will fluctuate from quarter to quarter and from year to year depending on, among other factors, demand for the Company's products, new product introductions and the mix of approved products shipped.

Research and development expense increased 28% to \$3.0 million in the three months ended June 30, 2004 compared to \$2.3 million for the three months ended June 30, 2003. The increase results from increased spending for two Phase III(b) trials for Xyrem, SXB-15 and EXCEEDS. The first trial, SXB-15, is complete and data from the trial was announced in May 2004. The second trial, EXCEEDS, is expected to be completed during the third quarter. The Company also recently announced that it has initiated patient enrollment in a clinical trial designed to evaluate Xyrem in the treatment of Fibromyalgia Syndrome. The Company expects that initial data from this 150 patient placebo-controlled trial will be available in mid-2005. Trial sites are located throughout the United States and Canada with approximately twenty participating centers. Clinical spending for trials is dependent on a number of factors, including among others, the number of human subjects screened and enrolled in the trial, and the number of active clinical sites.

Sales and marketing expense increased 45% to \$5.4 million for the three months ended June 30, 2004 from \$3.7 million for the three months ended June 30, 2003. This increase results from the implementation of various selling and marketing programs related to Xyrem.

General and administrative expense decreased 24% to \$1.0 million for the period ended June 30, 2004 compared to \$1.4 million for the three months ended June 30, 2003. The decrease is the result of expense reductions in staffing related to the product divestments in the prior year.

Because the Company recorded a loss for the three months ended June 30, 2004, no income tax expense was recorded. During the three months ended June 30, 2003, the Company recorded \$0.3 million of income tax expense related to the product dispositions. Since the Company expects to be unprofitable for at least the next several quarters, the Company continues to provide a valuation allowance for the entire amount of its net deferred tax assets.

### Six Months Ended June 30, 2004 vs. Six Months Ended June 30, 2003

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The net loss applicable to common shareholders was \$4.9 million for the six months ended June 30, 2004 compared to a net income applicable to common shareholders of \$21.9 million for the six months ended June 30, 2003. The Company divested three products in the second quarter of 2003 resulting in a net gain of \$30.3 million. Operating expenses increased over the prior year primarily due to the commercialization of Xyrem and the ongoing development activities for Xyrem.

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### Product Revenue Summary

The following is a summary of product revenue for the six months ended June 30, 2004 compared to the six months ended June 30, 2003:

000s	Six Months ended June 30,		Variance	
	2004	2003	\$	%
Antizol	4,512	3,136	\$ 1,376	44%
Antizol-Vet	124	120	4	3
Cystadane	785	666	119	18
Xyrem	3,977	1,356	2,321	193
Busulfex (1)	--	3,433	(3,433)	(100)
Elliotts B (1)	--	15	(15)	(100)
Sucraid (1)	--	191	(191)	(100)
<b>Total</b>	<b>9,398</b>	<b>8,916</b>	<b>\$ 482</b>	<b>5%</b>

(1) These products were divested during the second quarter of 2003.

Revenue from products marketed during the six months ended June 30, 2004 increased 78% compared to the same products in the corresponding 2003 period. Total reported product revenue was \$9.4 million, an increase of \$0.5 million or 5% compared to reported revenue in the first half of 2003. Reported product revenue for the six months ended June 30, 2003 included \$3.6 million from products divested in 2003. Antizol increased from the prior year as a result of increased stocking and continued penetration of the number of poisonings or suspected poisonings treated. Xyrem increased over the prior year as a result of continued growth in new prescriptions and patient base.

Licensing revenue for the six months ended June 30, 2004 was \$1.4 million which includes a milestone payment related to the European licensing of Xyrem and an amortized portion of the upfront payment for the same agreement. The upfront payment is being amortized on a straight line basis over an 18-month period beginning April 2004.

Gross profit margins increased to 85% for the six-months ended June 30, 2004 compared to 84% for the same period the prior year due to product mix. Cost of sales was \$1.4 million for the six months ended June 30, 2004 compared to \$1.5 million for the same period the prior year. The three products divested in the second quarter of 2003 had slightly lower margins than the Company's current product portfolio. Cost of sales as a percentage of net sales will fluctuate from quarter to quarter and from year to year depending on, among other factors, demand for the Company's products, new product introductions and the mix of approved products shipped.

Product development expense increased 60% to \$7.2 million in the six months ended June 30, 2004 compared to \$4.5 million for six months ended June 30, 2003. The increase results from increased spending for two Phase III(b) trials for Xyrem, SXB-15 and EXCEEDS. The first trial, SXB-15, is complete and data from the trial was announced in May 2004. The second trial, EXCEEDS, is expected to be completed during the third quarter. The Company also recently announced that it has initiated patient enrollment in a clinical trial designed to evaluate Xyrem in the treatment of Fibromyalgia Syndrome. The Company expects that initial data from this 150 patient placebo-controlled trial will be available in mid-2005. Trial sites are located throughout the United States and Canada with approximately twenty participating centers. The Company expects that product development spending will increase to \$3.5 million to \$4.0 million per quarter in the second half of 2004. Clinical spending for trials is dependent on a number of factors, including among others, the number of human subjects screened and enrolled in the trial, and the number of active clinical sites.

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Sales and marketing expense increased 10% to \$8.8 million for the six months ended June 30, 2004 from \$8.0 million for the six months ended June 30, 2003. This increase results from the implementation of various selling and marketing programs. The Company expects sales and marketing spending for 2004 to be approximately 5% higher than the expense in 2003 due to medical education and other programs related to Xyrem.

General and administrative expense decreased 15% to \$2.3 million for the six-months ended June 30, 2004 compared to \$2.6 million for the six months ended June 30, 2003. The decrease from the prior year in general and administrative

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expenses was the result of staff reductions resulting from the product divestments in the second quarter of 2003. General and administrative expenses over the second half of 2004 are expected to be similar to the first half levels.

Because the Company recorded a loss for the six months ended June 30, 2004, no income tax expense was recorded. During the six months ended June 30, 2003, the Company recorded \$0.3 million of income tax expense related to product dispositions. Since the Company expects to be unprofitable for at least the next several quarters, the Company continues to provide a valuation allowance for the entire amount of its deferred tax assets.

### **Liquidity and Capital Resources**

Since July 2, 1994, the effective date it was spun-off from Chronimed, Inc., the Company has financed its operations principally from net proceeds of \$90.8 million from several public and private financings, interest income and product sales, including \$30.3 million of net proceeds from the divestiture of products during the quarter ended June 30, 2003.

The primary sources of capital for the six months ended June 30, 2004 were from product revenues, a milestone payment from a licensing partner, and existing balances of cash and cash equivalents. The Company also had proceeds from the exercise of existing stock options of \$0.7 million for the six months ended June 30, 2004.

In October 2003, the Company announced that it had licensed European sales and marketing rights for Xyrem (sodium oxybate) oral solution to Celltech Pharmaceuticals, a division of Celltech Group plc. Under the terms of the agreement, Celltech will be responsible for the registration, sales and marketing of Xyrem in Europe. The ten-year licensing agreement includes the use of Xyrem in narcolepsy and provides Celltech with rights to negotiate in regard to other potential future indications including fibromyalgia syndrome. The term of this agreement is for 10 years from the date of approval in Europe with automatic extension until Celltech provides 12 month notice to Orphan Medical. The agreement may be terminated under certain conditions including material breach of contract provisions prior to the ten year initial term. Celltech made an initial payment of \$2.5 million to the Company in October 2003 and may make further payments up to \$6 million tied to product development milestones and up to \$7 million tied to sales-related milestones should these milestones be met. According to the terms of the agreement, milestone payments are due within 30 days of achieving the milestone. In addition to the milestone payments, Celltech will also pay the Company a royalty on sales of the product which is expected to begin no earlier than 2005. During the first quarter of 2004, the Company received a milestone payment of \$1.0 million from Celltech based on the filing of the Xyrem application with the European authorities, an achievement triggering a product development milestone. The Company does not expect to receive a milestone payment during the third quarter of 2004; however, a \$1.0 million milestone payment may become due in the fourth quarter of 2004 if certain conditions are met. Subsequent to that time, the next milestone payment likely will not be due until the second quarter of 2005 at the earliest.

The Company used more capital than its operations generated for the six months ended June 30, 2004. The Company expects to incur a loss from operations in 2004 and 2005, which will continue to decrease its capital reserves. The Company continues to invest its capital in product development activities that may provide opportunities to enhance the commercial opportunities for Xyrem. The Company has committed \$7.1 million to future product development activities. In addition, the Company also continues to use capital to develop and enhance the commercial programs for Xyrem. The Company expects that these efforts may result in increased Xyrem revenues. The Company expects that its current cash balances, cash flow from product revenues and any milestone payments received in accordance with the terms of the Celltech agreement will be sufficient to fund operations beyond the first half of 2005; however, the Company may seek additional capital in order to fund operations or should it decide to expand its product development programs and acquire additional products.

On April 14, 2004, the Company filed a shelf registration statement with the Securities and Exchange Commission (SEC) for the registration of 4,000,000 shares of common stock. Although we believe we have sufficient cash available for currently anticipated clinical trials, proceeds of any stock sale might be used for operations, clinical trials related to products that we may acquire or develop in the future or for trials related to new indications of existing products. This registration statement is still under review and has not yet been declared effective by the SEC.

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The Company entered into a line of credit facility with a commercial bank on March 28, 2003. The facility was amended in June 2003 as part of the Company's product divestments in June. The line of credit facility, which had an initial term of one-year, includes a borrowing base equal to 75% of eligible accounts receivable up to a maximum

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amount of \$2.5 million as amended in June 2003. Certain other assets have also been pledged as collateral for this facility. The interest rate is equal to two points over the bank's prime rate, with a minimum rate of 6.75%. The Company will be subject to certain other requirements during the term of the facility, including minimum quarterly net equity amounts and maximum monthly net losses. In late March 2004, the Company extended this facility to September 30, 2004 under the same terms described above. The Company's borrowing capacity at June 30, 2004 was approximately \$1.7 million. The Company had not borrowed under this facility as of June 30, 2004.

The Company's commitments for outside development spending decreased to approximately \$7.1 million at June 30, 2004 from \$14.7 million at December 31, 2003. These commitments are generally made for periods shorter than one year. The decrease is principally attributable to the completion of the Phase III(b) clinical trials assessing Xyrem for the treatment of EDS in narcolepsy. This was offset by costs associated with the initiation of the Fibromyalgia trial, which began patient enrollment in the second quarter of 2004. The Company expects quarterly product development spending in the second half of 2004 to be in the \$3.5 million to \$4.0 million range depending on patient accession in the Fibromyalgia trial. In addition, the Company continues to assess opportunities relating to current products and to new product opportunities which, if pursued, will increase development spending. Due to the dependence of this estimate on the results of the studies and other variable components, actual results may be different than the Company's estimates.

In early July 2004, the Company received approximately \$1.9 million from the exercise of stock options. Management believes the Company's current cash availability, including the \$1.9 million received in early July, anticipated operating cash flows from product revenues and anticipated milestone payments from Celltech will be sufficient to fund its operations beyond the middle of 2005.

For continued listing on the NASDAQ National Market, a company must satisfy a number of requirements, which in the Company's case include either: (1) net equity in excess of \$10.0 million or (2) a market capitalization of at least \$50.0 million. The Company met both the thresholds at June 30, 2004. The Company's market capitalization was approximately \$109.3 million as of June 30, 2004 (based on the last sale price of \$10.03 and 10.9 million shares outstanding). Although the Company does not expect to be profitable in 2004, the Company nevertheless expects to continue to meet the listing requirements for listing on the Nasdaq National Market. However, there can be no assurance that the Company will continue to meet these requirements in the future.

In connection with the 1998 and 1999 private placements of convertible preferred stock, the Company agreed to certain restrictions and covenants, which could limit its ability to obtain additional financing. Even without these restrictions, the Company can make no assurances that additional financing opportunities will be available or, if available, on acceptable terms.

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### Geographic Sales Information

The Company tracks sales in two geographic regions, domestic and international. The Company has no assets outside of the United States. The following is a summary of net sales by geographic region for the three-month and six-month periods ended June 30, 2004 and 2003, respectively.

	(in thousands)			
	Three months ended June 30,		Six months ended June 30,	
	2004	2003	2004	2003
Domestic	\$ 4,833	\$ 3,865	\$ 8,899	\$ 7,763

	(in thousands)			
International	162	484	499	1,153
Total	\$ 4,995	\$ 4,349	\$ 9,398	\$ 8,916

***Off-Balance Sheet Arrangements***

We do not participate in transactions or have relationships or other arrangement with an unconsolidated entity, which include special purpose and similar entities or other off-balance sheet arrangements.

**RISK FACTORS**

An investment in our common stock involves a number of risks, including among others, risks associated with companies that operate in the pharmaceutical industry. These risks are substantial and inherent in our operations and industry. Any investor or potential investor should carefully consider the following information about these risks before buying shares of common stock.

**We have a history of losses, which we expect to continue.**

We have been unprofitable since our inception in January 1993, with the exception of 2003 due to the divestment of three products. We expect operating losses at least through 2004 because anticipated gross profits from product revenues will not offset our operating expenses and additional spending to continue drug development activities. The amount of these losses may vary significantly from year-to-year and quarter-to-quarter. Our actual losses will depend on, among other factors, the timing of product development, regulatory approval, and market demand for our Food and Drug Administration approved products. We cannot assure you that we will ever generate sufficient product revenues to achieve profitability.

**We cannot be sure that future capital will be available to meet our expected capital requirements.**

Although we believe that we have sufficient capital to meet our current business objectives, we may need additional capital if we expand our business plans, if business conditions change or results of operations are not as expected. Adequate funds for our operations, continued development, and expansion of our business plans, whether from financial markets or from other sources, may not be available when needed on acceptable terms, or at all. If we issue additional securities your ownership may be diluted.

In addition there are restrictions on our ability to raise additional capital that are part of the terms of the sales of our preferred stock. On July 23, 1998, we completed the private sale to UBS Capital of \$7.5 million of Senior Convertible Preferred Stock. On August 2, 1999, we completed another private sale to UBS Capital of \$2.95 million of Series B Convertible Preferred Stock. In conjunction with the issuance of the preferred shares, we agreed to several restrictions and covenants, and granted certain voting and other rights to the holders of the preferred shares. One of the most important of these restrictions is that we cannot incur additional indebtedness, except for indebtedness secured solely by our trade receivables, until we have profitable operations, subject to certain limitations. Another important restriction is that, without the approval of a majority of the preferred stockholders, we cannot issue additional equity securities unless the selling price per share exceeds the then conversion price of the outstanding convertible preferred stock or the sale of equity is accomplished in a public offering. The present conversion price is \$8.14 per share for the

Senior Convertible Preferred Stock and \$6.50 for the Series B Convertible Preferred Stock. These restrictions could make it more difficult and more costly for us to obtain additional capital. We cannot assure you that additional sources of capital will be available to us or, if available, on terms acceptable to us.

**Possible Price Volatility and Limited Liquidity of Common Stock.**

There is generally significant volatility in the market prices and limited liquidity of securities of early stage companies, and particularly of early stage pharmaceutical companies. Contributing to this volatility are various factors and events that can affect our stock price in a positive or negative manner. These factors and events include, but are not limited to:

- general national and international economic and political developments
- announcements by us or our competitors of new product developments or clinical testing results;
- governmental approvals, refusals to approve, regulations or actions;
- developments or disputes relating to patents or proprietary rights;
- public concern over the safety of therapies;
- financial performance;
- fluctuations in financial performance from period to period; and
- small float or number of shares of our stock available for sale and trade.

There is also a risk that the market value and the liquidity of the public float for our common stock could be adversely affected in the event we no longer meet the NASDAQ's requirements for continued listing on the National Market. For continued listing on the NASDAQ National Market, a company must satisfy a number of requirements, which in our case includes either: (1) minimum net equity in excess of \$10.0 million as reported on Form 10-Q or Form 10-K or (2) a market capitalization of at least \$50.0 million. Market capitalization is defined as total outstanding shares multiplied by the last sales price quoted by NASDAQ. We met both criteria as of December 31, 2003 and June 30, 2004; however, we cannot assure you that the market capitalization threshold will continue to be met or that we will be able to generate adequate capital to meet the net tangible asset requirement.

These and other factors and events may have a significant impact on our business and on the market price of the common stock.

**There is a limited market for our products.**

Most orphan drugs have a potential United States market of less than \$25 million annually and many address annual markets of less than \$1 million. Although we expect combined revenue from the sales of Antizol, Cystadane, and Antizol-Vet in 2004 to be approximately \$7.0 million, we believe that the total market opportunity for these three products is not likely to exceed \$10.0 million in the foreseeable future.

We expect revenue from Xyrem in 2004 to be approximately \$11.7 million to \$12.7 million. Xyrem is indicated for the treatment of cataplexy in narcolepsy, and, if our clinical trials in our product development programs that are underway produce positive data, they may result in increased market opportunity for Xyrem. We cannot assure, however, you that sales of our products will be adequate to make us profitable even if the products are accepted by medical specialists and used by patients.

**We currently rely on the limited protection of the Orphan Drug Act for certain products.**

Since its inception, all of our products have been granted orphan drug status by the FDA. Medicines developed or acquired in the future may hold orphan drug status, although we may develop or acquire products that do not hold such status if we can obtain appropriate proprietary protection through patents or otherwise. Currently two of our products have orphan drug status: Xyrem with an expiration date of July 17, 2009 and Antizol, with an expiration date of December 6, 2006.

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**United States**

Under the Orphan Drug Act, the FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition. The Orphan Drug Act generally defines rare disease or condition as one that affects populations of fewer than 200,000 people in the United States. The Orphan Drug Act provides us with certain limited protections for our products.

The first step in obtaining the limited protection under the Orphan Drug Act is acquiring the FDA's approval of orphan drug designation, which must be requested before submitting a New Drug Application (NDA). After the FDA grants orphan drug designation, it publishes the generic identity of the therapeutic agent and the potential orphan use specified in the request. Orphan drug designation does not constitute FDA approval. In addition, orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory approval process.

The second step in obtaining the limited protection under the Orphan Drug Act is acquiring the FDA's recognition of orphan drug status. The Orphan Drug Act confers orphan drug status upon the first company to receive FDA approval to market a drug with orphan drug designation for a specific designated indication. Orphan drug status does not protect against another formulation or drug of materially different composition from being approved, with or without orphan drug status, for the same indication. FDA approval also results in United States marketing exclusivity for a period of seven years, subject to certain limitations. Although obtaining FDA approval to market a product with orphan drug status can be advantageous, we cannot assure you that the scope of protection or the level of marketing exclusivity will remain in effect in the



future. In addition, United States orphan drug status does not provide any marketing exclusivity in foreign markets. Although certain foreign countries provide development and marketing benefits to orphan drugs, we cannot assure you that such benefits can be obtained or, if obtained, will be of material value to us. The FDA has granted us orphan drug status for Xyrem, Antizol, and Cystadane. Upon expiration of orphan drug status, our products might be subject to competition from other pharmaceutical companies, with the exception of Xyrem in has some patent protection.

Even if the FDA approves an NDA for a drug with orphan drug designation, the FDA may still approve the same drug for a different indication, or a molecular variation of the same drug for the same indication. In addition, the FDA does not restrict doctors from prescribing an approved drug for uses not approved by the FDA for that drug. Thus, a doctor could prescribe another company's drug for indications for which our product has received FDA approval and orphan drug status. Significant off label use, that is, prescribing approved drugs for unapproved uses, could adversely affect the marketing potential of any of our products that have received orphan drug status and NDA approval by FDA.

The possible amendment of the Orphan Drug Act by Congress has been the subject of congressional discussion from time to time over the last ten years. Although Congress has made no significant changes to the Orphan Drug Act for a number of years, members of Congress have from time to time proposed legislation that would limit the application of the Orphan Drug Act. We cannot assure you that the Orphan Drug Act will remain in effect or that it will remain in effect in its current form. The precise scope of protection that orphan drug designation and marketing approval may afford in the future is unknown. We cannot assure you that the current level of exclusivity will remain in effect.

#### **Europe**

An orphan drug act was enacted in the European Union that provides up to ten years of market exclusivity for a drug that meets the requirements of the act. For a pharmaceutical product to qualify for the benefits of the act, the prevalence or incidence (whichever is greater) must not exceed five patients per 10,000 in the population. Our European partners have obtained orphan drug designation for Cystadane in Europe. The Company has obtained orphan drug designation for Xyrem and Antizol, for use in methanol poisonings, in Europe. European orphan drug designation of Antizol was withdrawn by the Company in 2003. We cannot provide assurance that any of our pharmaceutical products will qualify for orphan drug protection in the European Union or that another company will not obtain an approval that would block us from marketing our product in the European Union.

#### **Patents and other proprietary rights are important factors in our business.**

The pharmaceutical industry and the investment community place considerable importance and value on obtaining patent, proprietary, and trade secret protection for new technologies, products and processes. The patent position of

pharmaceutical firms is often highly uncertain and generally involves complex legal, technical and factual questions. Our success depends on several issues, including, but not limited to our ability:

- to obtain, and enforce proprietary protection for our products under United States and foreign patent laws; and other intellectual property laws;
- to preserve the confidentiality of our trade secrets; and
- to operate without infringing the proprietary rights of third parties.

We evaluate the desirability of seeking patent or other forms of protection for our products in foreign markets based on the expected costs and relative benefits of attaining such protection. We cannot assure you that any patents will be issued from any applications or that any issued patents will afford us adequate protection or competitive advantage. Also, we cannot assure you that any issued patents will not be challenged, invalidated, infringed or circumvented. Parties not affiliated with us have obtained or may obtain United States or foreign patents or possess or may possess proprietary rights relating to our products. We cannot assure you that patents now in existence or later issued to others will not adversely affect the development or commercialization of our products.

We believe that the active ingredients or compounds in our FDA-approved products, Cystadane, Antizol, Antizol-Vet, and Xyrem, are in the public domain and presently are not subject to patent protection in the United States. We have a patent with respect to our formulation of Xyrem oral solution and other patents pending or issued.

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We have orphan drug protection for Antizol and Xyrem, which provides proprietary protection against potential competition. We could, however, incur substantial costs asserting any infringement claims that we may have against others. Upon expiration of orphan drug status our products might be subject to competition from other pharmaceutical companies.

We seek to protect our proprietary information and technology, in part, through confidentiality agreements and inventors' rights agreements with our employees. We cannot assure you that these agreements will not be breached, that we will have adequate remedies for any breach, or that our trade secrets will not otherwise be disclosed to or discovered by our competitors. We also cannot assure you that our planned activities will not infringe patents owned by others. We could incur substantial costs in defending infringement suits brought against us. We also could incur substantial costs in connection with any suits relating to matters for which we have agreed to indemnify our licensors or distributors. An adverse outcome in any such litigation could have a material adverse effect on our business and prospects. In addition, we often must obtain licenses under patents or other proprietary rights of third parties. We cannot assure you that we can obtain any such licenses on acceptable terms, if at all. If we cannot obtain required licenses on acceptable terms, we could encounter substantial difficulties in developing, manufacturing or marketing one or more of our products.

### **The FDA must agree with investigational new drug applications prior to the initiation of clinical development programs.**

Prior to the initiation of a clinical development program, companies submit an investigational new drug application (IND) to the FDA. If the FDA notifies the submitting sponsor that the IND requires additional information or is not approvable, the potential development program may be significantly delayed or terminated. We cannot assure you that IND applications submitted by us to the FDA, including butamben, will proceed in a timely manner. Further, it is possible that FDA action may result in the termination of the potential development program.

### **Approval from the FDA and foreign regulatory authorities must occur before any new products or a new indication for an existing product we may develop can be commercially sold, including Butamben.**

Government regulation in the United States and abroad is a significant factor in the testing, production and marketing of our current and future products. Each product must undergo an extensive regulatory review process conducted by the United States Food and Drug Administration and by comparable agencies in other countries. Appropriate approvals must be obtained before we are able to market or promote a product. We must also receive regulatory approval for each new indication for a product prior to marketing for that indication. We cannot market any medicine we may develop or

license as a prescription product in any jurisdiction, including foreign countries, in which the product does not receive regulatory approval. The approval process can take many years and requires the expenditure of substantial resources.

We depend on external laboratories and medical institutions to conduct our pre-clinical and clinical analytical testing in compliance with good clinical and laboratory practices established by the FDA. The data obtained from pre-clinical and clinical testing is subject to varying interpretations that could delay, limit or prevent regulatory approval. In addition, changes in FDA policy for drug approval during the period of development and in the requirements for regulatory review of each submitted NDA could result in additional delays or outright rejection.

We cannot assure you that the FDA or any foreign regulatory authority will approve a regulatory marketing application in a timely manner, if at all, with respect to any products we develop. Generally, the FDA and foreign regulatory authorities approve only a very small percentage of newly discovered pharmaceutical compounds that enter pre-clinical development. Moreover, even if the FDA approves a product, it may place commercially unacceptable limitations on the uses, or indications, for which a product may be marketed. This would result in additional cost and delay to the extent that further studies are required to provide additional data on safety or effectiveness.

### **FDA approval does not guarantee financial success.**

Four of our currently marketed products have been approved for marketing by regulatory authorities in the United States and elsewhere. We cannot assure you that any of our products will be commercially successful or achieve the expected financial results as a result of limited markets for our products as discussed in the risk factor entitled, "There is a limited market for our products." We may encounter unanticipated problems relating to the development, manufacturing, distribution and marketing of our products. Some of these problems may be beyond our financial and technical capacity to solve. The failure to adequately address any such problems could have a material adverse effect on our business and our prospects. In addition, the efforts of government entities and third party payors to contain or reduce the costs of health care may adversely affect our sales and limit the commercial success of our products.

We cannot completely insulate our drug development portfolio from the possibility of clinical or commercial failures or generic competition. Some products that we have selected for development may not produce the results expected during clinical trials or receive FDA approval. Drugs approved by the FDA may not generate product sales of an acceptable level. We have discontinued the development of eleven products from our portfolio since inception.

In addition we continue to invest in the development of additional indications for Xyrem. This spending, along with costs associated with the on-going marketing and selling of Xyrem, resulted in a loss from operations in fiscal 2003. We expect that we will incur a loss from operations in 2004 and 2005.

**Significant government regulation continues once a product is approved for sale.**

After a reviewing division of the FDA approves a drug, the FDA's Division of Drug Marketing, Advertising and Communication must accept such drug's marketing claims, which are the basis for the drug's labeling, advertising and promotion. We cannot be sure that the Division of Drug Marketing, Advertising and Communication will accept marketing claims we propose to the agency. The failure of the Division of Drug Marketing, Advertising and Communication to accept our proposed marketing claims could have a material adverse effect on our business and prospects.

The FDA can require that a company conduct post-marketing adverse event surveillance programs to monitor any side effects that occur after the company's drug is approved for marketing. If the surveillance program indicates unsafe side effects, the FDA may recall the product, and suspend or terminate a company's authorization to market the product. The FDA also regulates the manufacturing process for an approved drug. The FDA may impose restrictions or sanctions upon the subsequent discovery of previously unknown problems with a product or manufacturer. One possible sanction is requiring the recall of such product from the market. The FDA must approve any change in manufacturer as well as most changes in the manufacturing process prior to implementation. Obtaining the FDA's approval for a change in manufacturing procedures or change in manufacturers is a lengthy process and could cause production delays and loss of sales, which would have a material adverse effect on our business and our prospects.

Certain foreign countries regulate the sales price of a product after marketing approval is granted. We cannot be sure that we can sell our products at satisfactory prices in foreign markets even if foreign regulatory authorities grant marketing approval.

**We rely on others for product development opportunities.**

We engage only in limited research to identify new pharmaceutical compounds. To build our product portfolio, we have adopted a license and acquisition strategy. This strategy for growth requires us to identify and acquire pharmaceutical products targeted at niche markets within our selected therapeutic markets. These products usually require further development and approval by regulatory bodies before they can be marketed. We cannot assure you that any such products can be successfully acquired, developed, approved or marketed. We must rely upon the willingness of others to sell or license pharmaceutical product opportunities to us. Other companies, including those with substantially greater resources, compete with us to acquire such products. We cannot assure you that we will be able to acquire rights to additional products on acceptable terms, if at all. Our failure to acquire or license any new pharmaceutical products, or our failure to promote and market any products successfully within an existing therapeutic area, could have a material adverse effect on our business and our prospects.

We have contractual development rights to certain compounds through various license agreements. Generally, the licensor can unilaterally terminate these agreements for several reasons, including, but not limited to the following reasons:

- for cause if we breach the contract;
- if we become insolvent or bankrupt;
- if we do not apply specified minimum resources and efforts to develop the compound under license; or
- if we do not achieve certain minimum royalty payments, or in some cases, minimum sales levels.

We cannot assure you that we can meet all specified requirements and avoid termination of any license agreements. We cannot assure you that if any agreement is terminated, we will be able to enter into similar agreements on terms as favorable as those contained in our existing license agreements.

We have invested most of our capital in the development of products already licensed to or under the control of the Company, therefore this risk has not had a material impact on our business in the past. As we look for additional opportunities to expand our product portfolio, this risk factor may have an adverse effect on our business.

**A failure by our manufacturers or suppliers to deliver product timely could adversely affect sales revenue.**

We do not have and do not currently intend to establish any manufacturing capability for drug products. Instead, we engage third parties to manufacture our products. Failure by parties with whom we contract to adequately perform their responsibilities may delay the submission of products for regulatory approval, impair our ability to deliver our products on a timely basis or otherwise adversely affect our business and our prospects.

The loss of either a bulk drug supplier or drug product manufacturer would require us to obtain regulatory clearance in the form of a pre-approval submission and incur validation and other costs associated with the transfer of the bulk drug or drug product manufacturing process. We believe that it could take as long as two years for the FDA to approve such a submission. Because our products are targeted to relatively small markets and our manufacturing production runs are small by industry standards, we have not incurred the added costs to certify and maintain secondary sources of supply for bulk drug substance or backup drug product manufacturers for some products. Should we lose either a bulk drug supplier or a drug product manufacturer, we could run out of marketable product to meet market demands or investigational product for use in clinical trials, while we wait for the FDA approval of a new bulk drug supplier or drug product manufacturer.

During the course of negotiations in the ordinary course of business to renew or extend an agreement with a manufacturing vendor, on occasion, the Company's vendors have indicated that if price increases cannot be

successfully negotiated, their agreement may need to be terminated. If this were to occur, we believe that there are alternate manufacturing and supply sources that would be available both on acceptable terms and on a timely basis for our products. In addition, our agreements generally require the manufacturer or supplier to continue to perform their obligations under these agreements for at least one year, and in some cases, two years, following formal notice of termination, during which period we would seek to implement new manufacturing and supply relationships. However, we cannot assure you that the change of a bulk drug supplier or drug product manufacturer and the transfer of the processes to another third party will be approved by the FDA, and if approved, in a timely manner. Therefore, we may experience additional costs and delay with switching providers, which in turn could adversely affect sales revenue.

***Bulk Drug Supply***

Bulk drug substance is the active chemical compound used in the manufacture of our drug products. We currently have a single supplier for the supply of bulk drug substance used in Cystadane, Antizol and Antizol-Vet. If we were to lose this company as a supplier, we would be required to identify a new supplier for the bulk drug substance. We also currently use a single supplier for the supply of bulk drug substance used in Xyrem, which is expected to account for approximately 65% of our revenue in 2004. If we were to lose this company as a supplier, we would be required to identify a new supplier.

***Drug Product Manufacture***

From bulk drug substance, drug product manufacturers formulate a finished drug product and package the product for sale or for use in clinical trials. We also use a single supplier for drug product manufacturing of Antizol, Antizol-Vet and a different supplier has been authorized to manufacture Xyrem. If we were to lose either of these companies as a manufacturer, we would be required to identify a new manufacturer; We cannot assure you that our drug product manufacturing arrangements with either or both of these suppliers will not change.

**We cannot control our contractors' compliance with applicable regulations.**

The FDA defines and regulates good manufacturing practices to which bulk drug suppliers and drug product manufacturers are subject. The Drug Enforcement Agency (DEA) defines and regulates the handling and reporting requirements for certain drugs which have abuse potential, known as scheduled drugs. Foreign regulatory authorities prescribe similar rules and regulations. Our supply and manufacturing contractors must comply with these regulatory requirements. Failure by our contractors to comply with FDA or DEA requirements or applicable foreign requirements could result in significant time delays or in our inability to commercialize or continue to market a product. Either result could have

a material adverse effect on our business and prospects. Failure to comply with good manufacturing practices or other applicable legal requirements can lead to federal seizure of violative products, injunctive actions brought by the federal government, or potential criminal and civil liability for Orphan Medical, our officers, or our employees. This risk has not impacted us in the past and we are not aware of any instances of noncompliance with applicable regulations that may materially impact our business. We cannot assure you that we will be able to maintain relationships either domestically or abroad with contractors whose facilities and procedures comply or will continue to comply with FDA or DEA requirements or applicable foreign requirements.

**We have a single distribution for three of our products: Antizol, Antizol-Vet and Cystadane.**

We have an agreement with a single distribution contractor to provide integrated distribution and operations services to support transactions between us and our wholesalers, specialty distributors, and direct customers. This contractor also provides reimbursement management, patient assistance and information hotline services and specialty distribution and marketing services to physician practices with respect to our products. The contractor currently distributes Antizol, Antizol-Vet and Cystadane. The contractor may also distribute future products should those products receive marketing clearance from the FDA. A failure by this distributor to fulfill its responsibilities might have an adverse affect on our ability to meet customer demand in a timely manner.

We cannot assure you that our distribution arrangements with these entities or other companies would be available, or continue to be available to us on commercially acceptable terms. The loss of a distributor or failure to renew agreements with an existing distributor would have a material adverse effect on our business and prospects.

**Xyrem is classified as a Schedule III controlled substance.**

We have an agreement with a specialty pharmacy to distribute Xyrem. Xyrem is classified as a Schedule III controlled substance and approved under Subpart H of the FDA's review process, and distribution is strictly controlled. The specialty pharmacy is the only source through which Xyrem can be obtained. Distribution is governed by the FDA's Subpart H regulations and complies with the risk-management controls jointly developed by Orphan Medical, the FDA, the Drug Enforcement Agency and law enforcement agencies. Every shipment of Xyrem is subject to stringent safeguards to ensure it reaches only individuals for whom it has been legitimately prescribed.

Our purchases of sodium oxybate, the active ingredient in Xyrem, for use in the production of Xyrem are subject to quotas that are published and approved by the U.S. Drug Enforcement Administration. Supply disruption could result from delays in obtaining DEA approvals or the receipt of approvals for quantities of sodium oxybate that are insufficient to meet current or projected product demand. The quota system also limits our ability to build inventories as a method of insuring against possible supply disruptions.

**We rely on foreign marketing alliances and have no assurance of foreign licensees.**

Our strategy to sell our products in foreign markets is to license foreign marketing and distribution rights to a foreign company after a new drug application is submitted or approved in the United States. We consider Europe, Asia, and Canada our most attractive foreign markets. Our current foreign arrangements are:

*Europe.* We have licensed the marketing and distribution rights for Xyrem and Cystadane in Europe. If our licensees are unsuccessful in their registration and distribution efforts, we may find it difficult to contract with other distributors for these products within Europe. Distribution of all products except Antizol is limited to named patient or emergency use basis until full regulatory approval is obtained. Antizol has been approved for use in the United Kingdom but is limited to named patient basis in other parts of Europe. This distribution of the Company's products is expected to result in a limited contribution to the Company's revenues.

*Australia and New Zealand.* We have licensed marketing and distribution rights for Cystadane in Australia and New Zealand, but sales of these products have not been material. We do not expect sales to increase in the near future to the point that they become material.

*Israel.* We have licensed marketing and distribution rights for Antizol and Cystadane in Israel. Full regulatory approval for Cystadane was obtained in Israel in February 2000. We do not expect such distribution to result in material revenues.

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*Canada.* We have licensed marketing and distribution rights for Antizol in Canada. For Cystadane we have only licensed the distribution rights in Canada. We do not expect such distribution to result in material revenues.

We depend on our foreign licensees for the regulatory registration of our products in foreign countries. We cannot be sure that our licensees can obtain such registration. In addition, we cannot be sure that we will be able to negotiate commercially acceptable license agreements for our other products or in additional foreign countries. Furthermore, we cannot assure you that these companies will be successful in negotiating acceptable pricing or in marketing and selling our products in their respective territories.

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### **Our products might be recalled.**

A product can be recalled at our discretion or at the discretion of the FDA, the U.S. Federal Trade Commission, or other government agencies having regulatory authority for marketed products. A recall may occur due to disputed labeling claims, manufacturing issues, quality defects, safety issues, or other reasons. We cannot assure you that a product recall will not occur. We do not carry any insurance to cover the risk of a potential product recall. Any product recall could have a material adverse effect on our business and prospects. To date, no recall of products marketed by the Company has occurred.

### **We face limits on price flexibility and third-party reimbursement.**

The flexibility of prices that we can charge for our products depends on government regulation, both in the United States and abroad, and on other third parties. One important factor is the extent to which reimbursement for our products will be available to patients from government health administration authorities, private health insurers and other third-party payors. Government officials and private health insurers are increasingly challenging the price of medical products and services. We are uncertain as to the pricing flexibility we will have with respect to, and if we will be reimbursed for, newly approved health care products.

In the United States, we expect continuing federal and state proposals to implement greater government control of the pricing and profitability of prescription pharmaceuticals. Cost controls, if mandated by a government agency, could decrease, or limit, the price we receive for our products or products we may develop in the future. We may not be able to recover our development costs, which could be substantial. We may not be able to realize an appropriate profit margin. This could have a material adverse effect on our business. Furthermore, federal and state regulations govern or influence reimbursement of health care providers for medical treatment of certain patients. We cannot assure you that action taken by federal and/or state governments, if any, with regard to health care reform will not have a material adverse effect on our business and prospects.

Certain private health insurers and third-party payors may attempt to control costs further by selecting exclusive providers of pharmaceuticals. If such arrangements are made with our competitors, these insurers and third-party payors would not reimburse patients who purchase our competing products. This would diminish the market for our products and could have a material adverse effect on our business and prospects.

### **We face intense competition in our industry.**

Competition in the pharmaceutical industry is intense. Potential competitors in the United States are numerous and include pharmaceutical, chemical and biotechnology companies. Many of these companies have substantially greater capital resources, marketing experience, research and development staffs and facilities than we do. We seek to limit potential sources of competition by developing products that are eligible for orphan drug status upon NDA approval or other forms of protection. We cannot assure you, however, that our competitors will not succeed in developing similar technologies and products more rapidly than we can. Similarly, we cannot assure you that these competing technologies and products will not be more effective than any of those that we have developed or are currently developing.

### **We expect rapid technological and other change to be constant in our industry.**

The pharmaceutical industry has experienced rapid and significant technological change as well as structural changes, such as those brought about by changes in health care delivery or in product distribution. We expect that pharmaceutical technology will continue to develop and change rapidly, and our future success will depend, in large part, on our ability to develop and maintain a competitive position. Technological development by others may result in our products becoming obsolete before they are marketed or before we recover a significant portion of the development and commercialization expenses incurred with respect to such products. In addition, alternative therapies, new medical treatments,

or changes in the manner in which health care is delivered or products provided could alter existing

treatment regimes or health care practices, and thereby reduce the need for one or more of our products, which would adversely affect our business and our prospects.

**We face substantial product liability and insurance risks.**

Testing and selling health care products entails the inherent risk of product liability claims. The cost of product liability insurance coverage has increased and is likely to continue to increase in the future. Substantial increases in insurance premium costs in many cases have rendered coverage economically impractical. We currently carry product liability coverage in the aggregate amount of \$30 million for all claims made in any policy year. Although to date we have not been the subject of any product liability or other claims, we cannot assure you that we will be able to maintain product liability insurance on acceptable terms or that our insurance will provide adequate coverage against potential claims. A successful uninsured product liability or other claim against us could have a material adverse effect on our business and prospects.

**Item 3. Quantitative and Qualitative Disclosures about Market Risk**

There have been no material changes to the Company's market risk since the filing of the Company's Annual Report on Form 10-K as amended.

**Item 4. Controls and Procedures**

*Evaluation of Disclosure Controls and Procedures.* Under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, we evaluated the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rule 13a-15(e) under the Securities Exchange Act of 1934, as amended (the Exchange Act)) as of the end of the period covered by this report. Based upon that evaluation, the Chief Executive Officer and Chief Financial Officer concluded that, as of the end of the period covered by this report, our disclosure controls and procedures are adequately designed to ensure that information required to be disclosed by us in the reports that we file or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in applicable rules and forms.

*Changes in Internal Controls.* During our second fiscal quarter, there were no significant changes made in our internal control over financial reporting (as defined in Rule 13(a)-15(f) under the Exchange Act) that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

**PART II OTHER INFORMATION**

**Item 1.** Legal Proceedings

Not Applicable

**Item 2.** Changes in Securities, Use of Proceeds and Issuer Purchases of Equity Securities

The Company did not repurchase shares during the quarter ended June 30, 2004.

**Item 3.** Defaults Upon Senior Securities

Not Applicable

**Item 4.** Submission of Matters to a Vote of Security Holders

The annual meeting of shareholders of Orphan Medical, Inc. was held on June 15, 2004. Matters submitted at the meeting for vote by the shareholders were as follows:

(a) Election of Directors.

The following directors were elected at the Annual Meeting, each with the following votes:

	<u>For</u>	<u>Withheld</u>
John Howell Bullion	10,799,182	112,077
Michael Greene	10,799,229	112,030
Julius A. Vida, Ph.D	10,797,329	113,930
William M. Wardell, M.D., Ph.D	10,693,044	218,215
Thomas B. King	10,693,245	218,014
Farah H. Champsi	10,693,345	217,914



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- (b) Approval of the 2004 Stock Incentive Plan and the authorization of 2,250,000 shares under the plan.

Shareholders approved the 2004 Stock Incentive Plan and authorization of 2,250,000 shares for issuance under the plan. A vote of 5,604,910 votes for, 1,319,857 votes against and 170 shares abstaining.

- (c) Ratification of Appointment of Independent Public Accountants.

Shareholders ratified the appointment of Ernst & Young LLP as the Company's independent public accountants for the fiscal year ending December 31, 2004, with a vote of 10,785,204 votes for, 91,620 votes against and 34,436 shares abstaining.

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### Item 5. Other Information

Not Applicable

### Item 6. Exhibits and Reports on Form 8-K

- (a) Exhibits:

<u>Exhibit Number</u>	<u>Description</u>
31.1	Certification of Chief Executive Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of Chief Financial Officer pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification of Chief Executive Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of Chief Financial Officer pursuant to Section 906 of the Sarbanes-Oxley Act of 2002

- (b) Reports on Form 8-K:

The Company filed the following Current Reports on Form 8-K during the quarter ended June 30, 2004:

- April 20, 2004 re: filing of S-3 registration statement with the SEC. Filed under items 5 and 7.
- April 22, 2004 re: announcement of quarterly results for the quarter ended March 31, 2004. Filed under item 12.
- May 24, 2004 re: results of SXB-15 clinical trial and subsequent announcement. Filed under item 5.

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**SIGNATURE**

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the Registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Orphan Medical, Inc.  
Registrant

Date: August 9, 2004

By: /s/ Timothy G. McGrath

Timothy G. McGrath  
Chief Financial Officer  
(duly authorized officer and  
principal  
financial officer)

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