ORPHAN MEDICAL INC
Form 10-K
April 01, 2002

SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-K

(MARK ONE)

[X] ANNUAL REPORT PURSUANT SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934 [NO FEE REQUIRED]

FOR THE FISCAL YEAR ENDED DECEMBER 31, 2001

OR

[ ] TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934 [NO FEE REQUIRED]

FOR THE TRANSITION PERIOD FROM TO

COMMISSION FILE NUMBER 0-24760

ORPHAN MEDICAL, INC.
(Exact name of registrant as specified in its charter)

DELAWARE	41-1784594
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification Number)
13911 RIDGEDALE DRIVE, SUITE 250 MINNETONKA, MN 55305 (Address of principal executive offices and zip code)	(952) 513-6900 (Registrant's telephone number, including area code)

SECURITIES REGISTERED PURSUANT TO SECTION 12(B) OF THE ACT: NONE

SECURITIES REGISTERED PURSUANT TO SECTION 12(G) OF THE ACT: COMMON STOCK, $.01 PAR VALUE

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 [X] No [ ]

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the

best of Registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. [ ]

Aggregate market value of common stock held by non-affiliates of Registrant, based upon the last sale price of the Common Stock reported on the Nasdaq National Market tier of The Nasdaq Stock Market on March 18, 2002 was $133,768,000. Common stock outstanding at March 18, 2002 was 10,290,000 shares.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the Registrant's Definitive Proxy Statement filed with the Securities and Exchange Commission in connection with the solicitation of proxies for the Registrant's Annual Meeting of Stockholders to be held on May 23, 2002 are incorporated by reference in Part III, Items 10, 11, 12 and 13 of this Form 10-K.

PART I.

This Annual Report on Form 10-K contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. All forward-looking statements are inherently uncertain as they are based on current expectations and assumptions concerning future events or future performance of the Company. Readers are cautioned not to place undue reliance on these forward-looking statements, which are only predictions and speak only as of the date hereof. Forward-looking statements 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, and are subject to numerous known and unknown risks and uncertainties. Actual results could differ materially from those currently anticipated due to a number of factors, including those identified in the section entitled "Risk Factors" in this Annual Report on Form 10-K, and in the Company's other filings with the Securities and Exchange Commission. The Company undertakes no obligation to update or publicly announce revisions to any forward-looking statements to reflect future events or developments.

Antizol(R), Antizol-Vet(R), Caprogel(TM), Busulfex(R), Intrachol(TM), Cystadane(R), Elliotts B(R) Solution, Sucraid(R), Xyrem(R), MedExpand(TM) , "The" Orphan Drug Company(TM), Orphan Medical, Inc.(R) and Dedicated to Patients with Uncommon Diseases(R) are trademarks of the Company.

ITEM 1. BUSINESS

OVERVIEW

Orphan Medical, Inc. (the "Company") acquires, develops and markets pharmaceutical products of high medical value for patients within selected therapeutic areas. A pharmaceutical product has high medical value if it offers a major improvement in the safety or efficacy of patient treatment. The Company currently concentrates its efforts on drugs with high medical value that may be marketed within three therapeutic areas: Antidotes, Oncology Support, and Sleep Disorders. Antizol and Busulfex are available commercially and are the Company's lead products in its Antidotes and Oncology Support therapeutic areas, respectively. Xyrem is an investigational drug and is expected to be the Company's lead product in its Sleep Disorders therapeutic area. In addition, the Company manufactures and distributes Cystadane and Sucraid that treat two rare congenital diseases. Antizol-Vet is marketed through separate channels to the

veterinary community. Although Cystadane, Sucraid, and Antizol-Vet do not fall into the selected therapeutic areas, the Company offers and expects to continue offering these products because they have high medical value and require limited resources to market and distribute.

Each of the Company's target therapeutic areas is characterized by a well-defined patient population that is treated by a well-defined group of medical specialists. The Company believes this targeted marketing approach makes a large sales force unnecessary to market the Company's products because marketing efforts can be focused on a limited number of medical specialists or patients. The high medical value of the Company's products facilitates marketing efforts directed toward users and prescribers. The Company intends to promote awareness of the key advantages of Orphan Medical branded products within Antidotes, Oncology Support, and Sleep Disorders for marketed products through its marketing and sales efforts. The Company's marketing and sales efforts differentiate its products on the basis of quality, potentially improved medical outcomes, and cost effectiveness. The Company uses established distributors of pharmaceutical products for its currently marketed products in a manner similar to that of most other pharmaceutical companies.

The Company believes its approach to pharmaceutical product development reduces the time, costs and risks traditionally involved in bringing pharmaceutical products to market. In general, the Company does not conduct basic research and does not attempt to discover new drugs. The Company's strategy is to acquire licenses to develop new products, or develop existing known therapeutic substances for new indications, and market products that preferably have existing clinical data that indicate therapeutic value and safety. In addition, the Company considers acquiring products that have already received marketing approval from the U.S. Food and Drug Administration (the "FDA"). The Company uses contract development, manufacturing, and consulting companies to assist it in its product development activities.

The Company operates within a single industry segment: pharmaceutical product development, marketing and sales. To date, the Company has obtained marketing approval from the FDA for six New Drug Applications ("NDA").

The Company's products are commercially available in the United States and several foreign countries. Revenues from sales of the Company's approved products within the United States were approximately 84.9% of total 2001 revenues, and revenues from sales outside the United States were approximately 15.1% of total 2001 revenues.

A Treatment Investigational New Drug ("IND") application for Xyrem was approved by the FDA in December 1998 and the Company began shipping Xyrem in February 1999 for use in its Treatment IND clinical trials. The Treatment IND allows the Company to seek payments for Xyrem used by patients enrolled in the Treatment IND clinical trials and to obtain additional clinical safety data for Xyrem. The Company submitted its NDA for Xyrem on October 2, 2000. The NDA was granted priority review status by the FDA, meaning the FDA had a goal of reaching a decision to either approve or disapprove or delay a decision regarding the NDA within 180 days of submission. On March 2, 2001, the FDA granted a 90 day extension to the Company's NDA in order that the Company could submit additional data requested by the FDA. On June 6, 2001, the FDA held a meeting of the Peripheral and Central Nervous System Advisory Committee to consider Xyrem. The Advisory Committee voted affirmatively that Xyrem was effective in treating cataplexy, but was split on the issue of Xyrem's safety due to the relatively small size of the safety database. As a result, the FDA issued an Approvable Letter for Xyrem on July 2, 2001. The Approvable Letter defined issues that required resolution before approval could be granted for the

treatment of cataplexy. On October 23, 2001, the Company announced that the FDA had accepted the Company's response, in the form of an amendment, to the Approvable Letter. The amendment represented the Company's complete response to the issues contained in the FDA's Approvable Letter. The amendment includes revisions to product labeling and the risk management program, a safety update of ongoing clinical trials, and respiratory data collected during all night polysomnographic recordings of narcolepsy patients in a clinical trial completed in late 2000. The Company believes it has addressed all questions and issues the FDA may have regarding the NDA for Xyrem. The FDA assigned April 9, 2002 as its action deadline for this NDA.

As a growing specialty pharmaceutical company, the Company did not achieve profitability in 2001 and does not expect to do so in 2002. The Company has significant capital requirements to support product development and marketing efforts until profitability is achieved. In December 2001, the Company completed the sale of 1,706,999 newly issued shares of common stock yielding net proceeds of $13.0 million.

STRATEGIC BACKGROUND

In the 1950s and early 1960s, drug development was relatively inexpensive and regulatory approval was straightforward. Pharmaceutical companies marketed their products through sales forces directly to physicians who generally had independent responsibility for prescription and purchase decisions. In the 1970s, however, regulatory standards and competition increased and the price of research and development and manufacturing rose dramatically. In the 1980s and 1990s, prescription decisions by physicians were constrained by managed care entities and drug companies revised their targeted rates-of-return or financial "hurdle rates", as well as other selection criteria, to avoid developing drugs whose incremental profit contributions were considered insufficient to provide acceptable returns on investment. Many of the drugs that did not meet these criteria were drugs that treat diseases affecting smaller patient populations. As a consequence, new drugs for such diseases were less likely to be developed by larger companies. Some research institutions, universities and small companies, however, have continued to develop and conduct clinical trials on these types of drugs.

The Company's business strategy is based on several factors relating to these and other changes in the health care and pharmaceutical industries:

- Larger pharmaceutical companies generally have increased their financial return rates, seek new products with annual revenues greater than $300 million and avoid developing new products that address diseases outside their therapeutic areas of focus. As a result, the Company believes many developmental products of high medical value are available for licensing. If these products are intended

to address smaller markets, they may be eligible for orphan drug designation. Many of these products have already been developed to the point where the time and cost required to bring the product to market can be reasonably estimated.

- The knowledge and skills required to address many aspects of drug development are available on a contract basis from outside companies or individuals.

- To address rapidly changing market forces, alternative means of marketing and distributing pharmaceuticals have been created. The advent of the Internet has dramatically increased the availability of information, including information related to health and health care products. Many products, particularly those targeted to smaller, well-defined markets,

do not require a large, specialized sales force and can be marketed through direct means such as exhibits at professional meetings, direct mailings, telemarketing, continuing medical education programs, and the Internet. In addition, these products can be effectively distributed through companies that are proficient in distribution of pharmaceutical products to smaller patient populations.

In response to these changes, the Company has adopted a business strategy that is centered on products of high medical value within well-defined strategic therapeutic areas. The Company uses the knowledge and skills available on a contract basis where necessary, and uses alternative marketing and distribution channels.

BUSINESS STRATEGY

The Company focuses on products of high medical value intended to address inadequately treated or uncommon diseases within selected therapeutic areas. A drug has high medical value if it offers a major improvement in the safety or efficacy of current patient treatments. In addition to products with high medical value, the Company seeks pharmaceutical products that have readily measured clinical endpoints, existing positive clinical data, proprietary attributes, eligibility for insurance reimbursement, and that offer attractive financial returns. The Company generally does not conduct basic research to discover new drugs, but instead seeks to acquire and further develop products that already have some data that indicate the presence of therapeutic value and safety. The Company's strategy of developing and marketing high medical value drugs with these clinical characteristics is intended to achieve the following benefits:

- Regulatory Requirements and Review -- Drugs of high medical value have a greater likelihood of receiving expedited review by the FDA. If these drugs also have smaller patient populations, the number of patients required for clinical trials is generally reduced.

- Product Development Time and Cost -- The Company attempts to concentrate resources and project management attention on a single medical indication in order to limit the amount of clinical information required by the FDA to clear a product for marketing. The time and cost of development is directly related to the amount of clinical information required for regulatory approval.

- Limited Infrastructure -- The Company believes that high quality pharmaceutical products can be developed efficiently and economically using well established independent contractors directed by its experienced staff. Accordingly, the Company uses the available pool of contract development, manufacturing, distribution and consulting companies to assist in product development and marketing activities. This approach allows the Company to avoid the costs, time and financial risks associated with developing an extensive infrastructure to perform these functions internally.

- Marketing Strategy -- To assess the viability potential product, the Company considers questions such as these:

  * Are there unmet therapeutic needs as defined by the customer (the patient or the health care practitioner)?

  * Are there other product development or product acquisition opportunities that complement the product or does the product fit in the Company's selected therapeutic areas of focus?

  * Can the product's brand be established and command significant market

share?

- Direct Sales -- The Company has built a small, specialized sales force to promote Antizol and Busulfex. The sales force has extensive knowledge of the Antidote and Oncology Support therapeutic areas, as well as extensive marketing and business experience. The Company's sales force utilizes a consultative, customer-oriented approach to selling. The Company expects to utilize a similar sales force approach with the Sleep Disorders therapeutic area, but will also use a contract sales organization to supplement the efforts of its sales force.

- Alliances -- The high medical value of the Company's products has interested other companies seeking to market the Company's products outside the United States. To date, the Company has agreements with nine companies relating to five of the Company's products. The Company also believes that its relationships with these and other partners, may provide strategic benefits, possibly in the area of product acquisition opportunities or in market share penetration programs.

- Attraction of Potential New Products -- As the Company's strategy and focus on pharmaceutical products of high medical value within a selected therapeutic area becomes better known and understood by others in the research and development community, and as the Company further proves its ability to market and sell into a therapeutic area, the Company expects more product development or acquisition opportunities will be presented to it in the future.

RISK MANAGEMENT

The Company's strategy has been designed, in part, to manage its overall business risk. The Company has pursued three distinct therapeutic areas rather than concentrating its resources on a single therapeutic area or a single platform technology. To reduce its product development risk, the Company generally seeks to develop products that (1) are known to the medical community and to the FDA, (2) have a straightforward formulation that can be readily manufactured with established technologies, and (3) do not require excessively specialized processes for development or manufacture. In addition, the Company generally seeks to acquire products that are already in Phase II or Phase III clinical trials, or in an earlier stage of development with proof of concept established. When a product is licensed without the equivalent of Phase II or III data, the Company may conduct one or more "proof of concept" to better assess the likelihood of efficacy or safety. Each such pilot trial is narrowly defined and has a separate budget that to date has not exceeded $500,000. The Company does not conduct extensive basic research to discover new chemical entities. The Company may also purchase rights to approved products. To reduce its marketing risk, the Company generally attempts to obtain some form of proprietary protection, such as orphan drug status, patent protection, exclusive licensing agreements, predominant market penetration or sole supplier agreements.

PROPRIETARY RIGHTS

The Company believes it is important that its products receive patent protection, orphan drug status or possess other attributes that limit potential competition. When available and appropriate, the Company will seek orphan drug status to enhance or provide proprietary protection to a product. A drug that has orphan drug designation and which is the first product to receive marketing approval for its product claim, indication or application, receives orphan drug status and is entitled to a seven-year exclusive marketing period in the United

States for that product claim, indication or application, subject to certain limitations. The Company currently has five products with orphan drug status. Applications for Orphan Drug designation will be made where appropriate and available for any additional indications or products that may be licensed in the future.

To encourage the continued development of drugs for smaller patient populations, the federal government enacted the Orphan Drug Act of 1983. This Act provides incentives to companies to develop and market drugs for diseases or conditions that are known to affect fewer than 200,000 people in the United States. A company must request orphan drug designation before an NDA is approved, and after the FDA grants orphan drug designation, the generic identity of the therapeutic agent (drug) and its potential orphan use (market) are published by the FDA. Orphan Drug designation does not convey any advantage in, or shorten the duration of, the regulatory approval process. The FDA may grant Orphan Drug designation to more than one company of an identical drug for the same designated indication, however under current law, orphan drug status is granted

to the first company receiving FDA marketing approval for a specific indication of a drug with orphan drug designation. A company receiving orphan drug status for a designated indication is entitled to a seven-year exclusive marketing period in the United States for the drug with the approved indication, subject to certain limitations. Orphan drug status, however does not prevent subsequent approval of a different drug for the same designated indication, nor subsequent approval of the same drug for a different designated indication, nor provide any marketing exclusivity in foreign markets. Since 1983, the FDA has assigned orphan drug designation to more than 650 potential products. Orphan Drug protection is available in Japan and the European Union under requirements similar to those in the United States.

The license agreement pursuant to which the Company has acquired rights to develop and market Busulfex provides for an assignment of the licensor's proprietary rights, including patent and technology rights. With respect to additional products it may license in the future, if any, the Company expects that such licenses will include, if such rights are available, an assignment of the licensor's proprietary rights with respect to the licensed product. Foreign patent applications have been filed for Busulfex and Xyrem. In 2000, the Company obtained the rights to patents in the United States and Europe covering the use of gamma hydroxbutyrate ("GHB") in the treatment of fibromyalgia. The Company has licensed the rights to two patents related to a new potential development opportunity. The Company is evaluating a development program for this product. The Company evaluates the desirability of registering approved patents or other forms of protection for its products in individual foreign markets based on the expected costs and relative benefits of attaining such protection.

THE REGULATORY PROCESS

Pharmaceutical products intended for therapeutic use in humans are governed by extensive FDA regulations in the United States and by comparable regulations in foreign countries. The process of seeking and obtaining FDA approval for an unapproved new human pharmaceutical product generally takes many years and involves the expenditure of substantial resources and considerable risk.

The process before a drug product can be marketed in the United States includes (i) pre-clinical laboratory and animal safety tests, (ii) the submission to the FDA of an IND application, (iii) clinical and other studies to assess safety and parameters of use, (iv) adequate and well-controlled clinical trials to establish the safety and effectiveness of the drug product, (v) the

submission to the FDA of an NDA, (vi) FDA approval of the NDA prior to any commercial sale or shipment of the product and (vii) marketing of the drug.

Upon the successful completion of clinical testing, a marketing application (e.g., NDA) is submitted to the FDA for approval. This application requires detailed data on the results of pre-clinical testing, clinical testing and the composition of the product; specimen labeling to be used with the drug; information on manufacturing methods; and samples of the product. Since the passage of the Prescription Drug User Fee Act ("PDUFA"), the FDA typically takes from six to eighteen months to review an NDA after it has been accepted for filing. Following its review of a marketing application, the FDA invariably raises questions or requests additional information. The NDA approval process can, accordingly, be very lengthy. Further, there is no assurance that the FDA will ultimately approve an NDA. The FDA can also determine that a drug is "approvable" contingent on satisfactory review of additional information requested by the FDA. If the FDA approves the NDA, the new product may be marketed for the applications or treatments that have been approved by the FDA. The claims with which a product can be marketed are also subject to review and approval by the Division of Drug Marketing, Advertising and Communications ("DDMAC"), the FDA's marketing surveillance department within the Center for Drugs. The FDA often clears a product for marketing with a modification, or restriction to the proposed label claims or requires that post-marketing surveillance, or Phase IV testing, to be conducted. The method and system of a drug's distribution can also be controlled by the FDA if approved under Subpart H regulations.

OPERATING FUNCTIONS

The Company has structured its operating functions to support its business strategy. Following is a general explanation of the typical steps in the Company's processes of product acquisition, development and marketing.

PRODUCT ACQUISITION

The Company actively searches for product licensing opportunities. The continual acquisition of products for development and/or commercialization is a key element of the Company's growth strategy. The Company attracts product acquisition proposals through a network of customer and industry contacts, licensing brokers and a growing awareness of its activities by governmental, academic and industry sources. Since its inception, the Company has evaluated many product opportunities. To date, sixteen products have been acquired, with seven of these products currently under development or being marketed.

The Company seeks to acquire pharmaceutical products within selected therapeutic areas that, in the Company's opinion, generally:

- Are of high medical value as defined by the customer (physician or patient) within a therapeutic area;
- Treat diseases that affect distinct patient populations;
- Are prescribed by physician specialists;
- Can be marketed with a relatively small, specialized sales team to health care specialists, health care institutions, and patients;
- Are likely to be eligible for reimbursement by third-party payors;
- Have or are candidates for patent protection, orphan drug designation or

have other characteristics that enhance the Company's competitive position;

- Treat diseases that have clinical endpoints (i.e., signs or symptoms) that are readily measured;

- Are conventional pharmaceutical products that are relatively straightforward in formulation and development, and do not involve the application of new technologies;

- Are in Phase II or Phase III clinical trials and have a relatively high likelihood of obtaining the approval of the FDA within three to four years of acquisition;

- Offer attractive potential financial returns with relatively inexpensive development costs; and

- Compliment other products in an existing therapeutic area or can be grouped with other products to build a new therapeutic area.

In selecting products for potential inclusion in its portfolio, the Company focuses on acquiring rights to medicines that serve niche or defined patient populations. Major drug companies are less likely to focus on these niche markets because they do not believe these markets will produce acceptable revenues and returns. This reluctance limits the number of potential sources of competition. In addition, a product designed for smaller patient populations is often eligible for orphan drug designation. By obtaining orphan drug designation, the Company is granted exclusive marketing rights in the United States for seven years, subject to certain limitations, after an NDA for a product is approved, if the Company is the first to receive approval for the designated drug and indication.

The Company seeks to acquire potential products that already have, or will not require, a substantial quantity of clinical data to demonstrate their relative efficacy and safety. The Company also searches for product candidates that represent new delivery methods or dosage forms of previously approved or known compounds because the Company believes these types of products are more likely to be quickly approved by the FDA and accepted by the medical community. In addition, the Company attempts to develop medicines where clear clinical endpoints can demonstrate their effectiveness. Generally, the Company seeks to acquire products that can be developed to the point of FDA approval within three to four years of their acquisition. The Company also focuses its development efforts on one indication and, when possible, one dosage form to

minimize development costs. Potential additional indications or dosage forms will only be evaluated after the primary NDA is submitted or approved.

An additional element of the Company's product development strategy is to acquire products that have proprietary protection or for which the Company can obtain some degree of proprietary protection. The Company seeks to accomplish this goal by selecting products that are eligible for Orphan Drug designation, are covered by patents or are the subject of an exclusive license from a sole supplier or a manufacturer with specialized or proprietary processes. The availability or likelihood of adequate levels of reimbursement from third-party payors is also an important factor in product acquisition decisions.

Company management reviews new product opportunities and makes recommendations to the Board of Directors regarding the license or acquisition of additional products. The product review process generally involves

discussions with customers (physicians and patients) within a specific therapeutic area to identify unmet needs, and with the initial product developer and experts in the disease treated by the drug; review of research publications and other databases to gauge competitive activities; market research aimed at identifying potential acceptance by the end user; technical evaluations to determine manufacturability and cost; expected FDA regulatory requirements to obtain acceptable marketing or promotional claims; and a legal review of any relevant proprietary rights. If this review indicates that the proposed product meets the Company's selection criteria, efforts to negotiate a license are initiated. Generally, the Company seeks to obtain licenses that require a minimal signing fee, are subject to commercially acceptable royalty levels and, where appropriate, provide for the continued involvement of the original developer in the development process through a consulting or similar arrangement.

The Company is continually seeking and evaluating additional proposed products within the selected therapeutic areas. Should the Company be unsuccessful in acquiring additional proposed products for development and commercialization within a selected therapeutic area, the Company may reassess the viability of the selected therapeutic area and redefine the selected therapeutic area to expand the number of potential products that might satisfy the Company's acquisition criteria for a viable therapeutic area to ensure adequate future growth of the Company.

PRODUCT DEVELOPMENT

Pharmaceutical product development is one of the Company's principal activities. The Company has incurred $41.5 million in research and development expense through December 31, 2001. In addition, the Company estimates that it will incur at least an additional $5.0 million in research and development expense relating to the six products it currently markets, obtain FDA approval for marketing Xyrem and to begin evaluation of additional Xyrem indications.

A major element of the Company's product development strategy is to use third-parties or contract research organizations (CROs) to assist in the conduct of safety and efficacy testing and clinical studies, to assist the Company in guiding products through the FDA review and approval process, and to manufacture and distribute any FDA approved products. The Company believes that maintaining a minimal infrastructure will enable it to develop products efficiently and cost effectively. To facilitate this strategy, the Company uses a team approach to develop its proposed products. A development team is organized and managed by senior staff. The development team is cross-functional and includes in-house experts, as well as appropriate outside consultants, to manage all development activities as well as market planning with respect to a proposed product. A development team designs a development plan, which will support the proposed indication and marketing claims, and creates and manages a financial budget for the proposed product and contracts with outside development agents and consultants to arrange for the necessary clinical and toxicology studies, manufacturing arrangements and FDA filings. Upon approval of the NDA by the FDA, the Company's marketing and sales staff manages marketing and sales of the proposed product.

The Company believes the use of third parties to develop and manufacture its products has several advantages. This approach allows a greater pool of resources to be concentrated on a product than if these functions were performed by internal personnel who were required to support all of the Company's products. Although this approach allows the Company to avoid the expense associated with developing a large internal

infrastructure to support its product development efforts, it results in the

Company being dependent on the ability of outside parties to perform certain critical functions. Over time, the Company expects to build internal capabilities to replace certain functions currently being performed by outside parties.

The Company's approach to product development requires project management by professionals with substantial industry experience. The Company believes it has in-house experts in areas of critical importance to all of its proposed products who can be consulted by the development teams. These areas include regulatory affairs, marketing and sales, quality assurance, manufacturing, clinical trials management, finance, information systems and general management.

The product development process is designed to identify problems associated with a proposed product's clinical aspects. The Company attempts to reduce the risk that a proposed product will not be accepted in the marketplace by conducting market research and defining commercial strategy with a product's development. A drug development portfolio cannot be completely insulated from potential clinical and marketing failures. It is likely that some proposed products selected for development by the Company will not produce the clinical or revenue results expected. To date, the Company has discontinued development activities with respect to eleven proposed products because the estimated financial returns of these proposed products were deemed unacceptable by management.

MANUFACTURING

The Company does not have and does not intend to establish any internal product testing, drug or chemical synthesis of bulk drug substance, and manufacturing capability for drug product. Manufacturers of the Company's products are subject to applicable Good Manufacturing Processes ("GMP") as required by FDA regulations, or other rules and regulations prescribed by foreign regulatory authorities. The Company is negotiating or has entered into bulk drug supply and drug product manufacturing agreements with third parties for all of its FDA approved products and is dependent on such third parties for continued compliance with GMP and applicable foreign standards. The Company believes that qualified manufacturers will continue to be available in the future, at a reasonable cost to the Company, although there can be no assurance that this will be the case.

Due to FDA mandated dating requirements and the limited market size for the Company's approved products, the Company may be subject to complex manufacturing logistics, minimum order quantities that could result in excess inventory as determined under the Company's accounting policy, unsalable inventory as a result of product expiring prior to use, and competition with others for manufacturing services when needed or expected. The Company has a production planning program to assess and manage the manufacturing logistics amongst the vendors supplying the required finished product components of bulk drug substance, drug product and packaging.

The Company is substantially dependent on its contract drug product manufacturers. These manufacturers have been approved by the FDA for the production of the Company's approved products. Following is a listing of the Company's contract drug product manufacturers:

CONTRACT DRUG PRODUCT MANUFACTURER	MARKETED AND PROPOSED PRODUCTS
An affiliate of Boehringer Ingelheim	Antizol, Antizol-Vet, Elliotts B Solution, and Busulfex
NutraMax, Inc.	Sucraid

Proclinical, Inc.	Cystadane
DSM Pharmaceuticals, Inc.	Xyrem (Treatment IND supplies)

In addition to the contract drug product manufacturers, the Company is substantially dependent on Ash Stevens, Inc. ("Ash Stevens") and Lonza, Inc. ("Lonza"). Ash Stevens is the Company's sole supplier of bulk drug substance for the manufacture of Antizol, Antizol-Vet, and Busulfex; while Lonza is the Company's sole supplier of bulk drug substance for the manufacture of Xyrem for use in clinical trials and is anticipated to be the sole supplier of any approved product.

MARKETING -- UNITED STATES

The Company has designed its product selection strategy to maximize the success of its marketing efforts. By having products that current and potential customers have identified as having "high medical value", the Company believes it should be able to more easily attract the attention of targeted segments of the medical community. The Company also believes that its focus on well-defined physician and patient populations will allow the use of a relatively small, focused sales force instead of a large, generalized sales force. Because of the distinct nature of most of its potential markets, the Company expects to be able to concentrate its marketing efforts on a limited number of medical specialists, or on patients themselves.

As part of its marketing efforts, the Company identifies and defines appropriate therapeutic areas, identifies customer needs within each therapeutic area, identifies specific product acquisition candidates within each therapeutic area, works with the development team to insure clinical data are collected that supports the desired indication and marketing claims, and if FDA approval is obtained, designs and implements marketing plans for each of its approved products. Market research is conducted to analyze the potential of products prior to their acquisition. Once a product is acquired and is being developed, further market research is completed and, based on this analysis, the product's marketing plan is developed. Upon submission of the NDA to the FDA, the product management responsibilities transition from the development team to the Company's marketing and sales staff. The development group continues to provide support where needed to enhance marketing and sales efforts. This group is responsible for all aspects of a product's marketing and sales, including product forecasting, positioning, price, promotion and physical distribution to successfully launch and commercialize the product. Senior sales and marketing employees lead a cross functional team of internal and external personnel to implement a product's marketing and commercialization plan. In addition, marketing and sales staff also supports the Company's international sales efforts through support of and interfacing with international partners.

MARKETING -- FOREIGN

In general, the Company expects to out-license foreign marketing, sales and distribution rights after an NDA is submitted or approved in the United States. The Company contracts with foreign companies (usually pharmaceutical companies) to market and distribute its products. The Company considers Europe and Asia to be its most attractive foreign markets. The Company has entered into marketing, sales and distribution agreements for Antizol, Cystadane and Sucraid in Europe, for Cystadane and Sucraid in Australia and New Zealand, for Busulfex, Cystadane, Elliotts B Solution and Sucraid in Israel, for Antizol and Cystadane in Canada, and for Elliotts B Solution in Central America. In October 1999, the Company signed a definitive agreement with Pierre Fabre Medicament, granting the French company exclusive rights to market and distribute Busulfex in 21 European

countries, as well as Argentina and South Africa. Upon approval, Pierre Fabre, a private company with sales in excess of $1 billion and approximately 8,300 employees, will market Busulfex to transplant centers through its 75 oncology marketing and sales personnel. The Company has an agreement with IDIS World Medicine to distribute Busulfex on a "named patient basis" to requesting physicians outside of the United States, Canada and Israel, and other approved products in territories where the Company's other products are not approved and where the Company does not have a distribution partner. The Company began shipments to IDIS in January 2000.

In December 2000, the Company signed a definitive agreement with Kirin Brewing Co., LTD to market and distribute Busulfex in Asia including Japan, the Peoples Republic of China, South Korea and Taiwan. Kirin is a diversified company with more than $14 billion worldwide revenues in the food and beverage industry. Kirin expanded into the pharmaceutical business in 1982 and has a strong presence in the fields of nephrology, cancer and cell production, immunology and allergy.

The Company's practice is to negotiate contracts with foreign distributors that generally provide for minimum order and sales performance. Minimum fees negotiated with foreign parties to date are not material and are not refundable, nor subject to future performance criteria. The foreign contracting party is responsible for obtaining marketing approval for the Company's product to which the agreement relates and the Company is responsible for providing selected U.S. regulatory information to the foreign party on request. The Company

cannot unilaterally terminate these agreements without established evidence of default, but these agreements do expire over a defined period of time and the Company may seek other foreign parties to provide comparable services upon expiration if not satisfied with the performance of its partners. The principal benefit a foreign party receives from entering into these agreements with the Company and paying the minimum fees, if any, is a contracted price for acquisition of product from the Company because the Company is the sole supplier of its approved products on a worldwide basis.

DISTRIBUTION

The Company does not currently intend to develop internal distribution capabilities because the Company believes its relatively low-volume products can be more economically and efficiently distributed through third party distribution organizations. Cystadane is principally distributed directly to patients through a third party mail order pharmacy, Chronimed, Inc. Elliotts B Solution, Antizol and Busulfex are primarily used in a hospital setting and are distributed by CORD Logistics, Inc, an affiliate of Cardinal Health. This distribution system allows the sale of these products directly into hospitals or, if customers prefer, through their primary wholesaler. Antizol-Vet is a product used in veterinary clinics and is distributed by CORD Logistics, Inc, an affiliate of Cardinal Health to individual veterinary clinics and a network of veterinary wholesalers.

COMPETITION

Potential competitors in the United States are numerous and include pharmaceutical, chemical and biotechnology companies. The Company experiences competition in several specific areas, including, but not limited to, those described below.

- Product Acquisition -- The Company competes with other entities in acquiring product rights from other companies, universities, other

research institutions, as well as from other potential licensors.

- Product Development Resources -- The Company competes for certain resources, such as the services of clinical investigators, contract manufacturers, advisors and other consultants. The Company will generally have little or no control over the allocation of such resources.

- Orphan Drug Designation -- The Company is aware of two other companies that have received orphan drug designation for products similar to two of the Company's products. Sparta Pharmaceutical (acquired by SuperGen in 1999) and Teva (formerly Biocraft) have been granted orphan drug designations for their intravenous busulfan and sodium oxybate, respectively. Intravenous busulfan and sodium oxybate are their equivalent of the Company's Busulfex and Xyrem products, respectively. The Company does not believe SuperGen is developing an intravenous busulfan. In 1999, the Company entered into an agreement with Teva, that, in effect, transfers Teva's development data to the Company. While the Company is not aware of others holding or seeking orphan drug designations for products that would compete with the Company's products for NDA approval, there can be no assurance that the Company's products will not have such competition for the protection conferred by orphan drug status.

- Marketing and Sales -- Each of the Company's current products will face competition from other products or from other therapeutic alternatives. In general, the Company's products will compete against products whose marketers have substantially greater resources, including large specialized sales forces, than the Company.

- Manufacturing -- The Company may also compete for limited manufacturing capacity or availability.

GOVERNMENT REGULATION

GENERAL

Political, economic and regulatory influences are subjecting the health care industry in the United States to fundamental change. Several potential approaches are under consideration, including mandated basic health care benefits, controls on health care spending through limitations on the growth of private health

insurance premiums and Medicare and Medicaid spending, price discounts from drug manufacturers, the creation of large purchasing groups and other significant changes to the health care delivery system. In addition, some states have adopted or are considering price controls and various health care reform proposals. The Company anticipates that Congress and state legislatures will continue to review and assess alternative health care delivery systems and payment methods and that public debate of these issues will likely continue in the future. Because of uncertainties regarding the ultimate features of reform initiatives and their enactment and implementation, the Company cannot predict which, if any, of such reform proposals will be adopted, when they may be adopted or what impact they may have on the Company or its prospects.

REIMBURSEMENT

Employers, through payments to their employee benefit plans, bear a significant share of the health care costs of their employees. These plans are typically administered by insurance companies, health maintenance organizations, preferred provider organizations and other third-party payors. Health care

services and products, including pharmaceutical products, are also paid for by government agencies, such as Medicaid. Employers and the payors involved in providing or administering health care benefits are increasingly turning to "managed care" systems to control health care costs. Under these systems, the administrative requirements and standards of care are established by the health care purchasers and providers and the benefit level depends on the negotiated price. Managed care systems usually limit treatment options to approved therapeutic regimens and "formularies," or lists of approved drugs and medical products.

Inclusion or listing on the formularies of managed care groups is important to the commercial success of a prescription medicine. A pharmaceutical must be included on a third-party payor's formulary or must be deemed "medically necessary" to be eligible for reimbursement by that payor. In deciding whether a drug is to be included on a formulary, payors will generally consider its therapeutic value and cost in comparison to other available treatments. The Company believes that the proprietary nature and medical usefulness of its products should assist it in its efforts to have its products approved for reimbursement. No assurance can be given, however, that the Company's products will be approved for reimbursement by third-party payors at acceptable levels, or at all.

PRODUCT APPROVALS

The Company's products require FDA approval in the United States and comparable approvals in foreign markets before they can be marketed. The development of investigational products and the marketing and supply of approved products require continuing compliance with FDA regulations on the part of the Company as well as its manufacturers and distributors.

SCHEDULED PRODUCTS

Products that are designated "controlled" substances also require compliance with regulations administered by the U.S. Drug Enforcement Agency ("DEA"), and similar regulations administered by state regulatory agencies. Xyrem's active ingredient is sodium oxybate, or GHB. PL 106-172, a public law which makes illicit GHB (gamma hydroxybutyrate) a Schedule I substance but designates FDA-approved GHB medicines Schedule III substances, was enacted in February 2000. Schedule I is the designation by which illegal and non-approved drugs are controlled. Schedule III includes additional handling and distribution requirements.

Each state has the ability to schedule products more strictly than the federally designated Schedule. Most states have adopted, either administratively or legislatively, the I/III schedule as described above. The Company continues its efforts to ensure consistency across all states by the time of commercial launch.

MANUFACTURING REGULATION

All facilities and manufacturing processes used to manufacture products for clinical use or sale in the United States must be operated in conformity with Good Manufacturing Practices (GMP). These represent the FDA requirements governing the production of pharmaceutical products. FDA approval is required before a contract manufacturer can implement most changes in manufacturing procedures for any of the Company's

approved products. The Company has established a quality assurance program to monitor third-party manufacturers of its products to promote compliance by such manufacturers with domestic and foreign regulations (based on country of use).

In addition, FDA approval is required to change contract manufacturers of approved products. Obtaining the FDA's approval for a change in manufacturing procedures or change in manufacturers could cause production delays and loss of revenue.

FOREIGN REGULATION

Products marketed outside of the United States are subject to regulatory approval requirements similar to those required in the United States, although the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary widely from country to country. No action can be taken to market any product in a country until an appropriate application has been approved by the regulatory authorities in that country. The current approval process varies from country to country, and the time spent in gaining approval varies from that required for FDA approval. In certain European countries, the price of a product must also be approved. The pricing review period often begins after market approval is granted. The Company intends to use foreign partners to apply for foreign marketing approvals.

INSURANCE

Providing health care products entails an inherent risk of liability. In recent years, participants in the health care industry have been subject to a large number of lawsuits alleging malpractice, product liability or related legal theories, many of which involve large claims and significant defense costs. The Company may from time to time be subject to such suits as a result of the nature of its business. The Company carries product liability insurance coverage in the aggregate amount of $20 million. The Company also carries a $10 million general business insurance policy. The Company does not carry any insurance to cover the financial risks associated with a potential FDA mandated recall of an approved product. There can be no assurance, however, that such insurance policies will be sufficient to fully indemnify the Company against any asserted claims or that such insurance will continue to be available.

HUMAN RESOURCES

The Company has fifty-eight full-time and eight part-time employees. The Company believes that its relationship with its employees is good. None of the Company's employees are represented by a labor union.

TRADE SECRETS

The Company also relies on trade secrets and proprietary know-how to protect certain of its technologies and potential products. The Company requires employees, consultants and advisors to enter into confidentiality agreements that prohibit disclosure to any third party or use of proprietary information, secrets and know-how for commercial purposes. Company employees also agree to disclose and assign to the Company all methods, improvements, modifications, developments, discoveries and inventions conceived during their employment that relate to the Company's business.

GRANTS

The FDA Office of Orphan Drug Products and the Small Business Administration offer grants to companies whose efforts meet certain requirements. From July 1, 1995 through December 31, 1998, the Company collected approximately $1,567,000 in grant proceeds with respect to approximately $1,567,000 in grant related disbursements for certain products. The grant proceeds collected by the Company are non-refundable. There can be no assurance that additional grants from these agencies will be made available to the Company in subsequent periods. The Company currently has no active grants.

DISCONTINUED DEVELOPMENT PRODUCTS

As of December 31, 2001, the Company has discontinued development activities on eleven proposed products. During 1997, the Company discontinued development activities on the following proposed products: Colomed, Caprogel, Clonidine, alpha-galactosidase A, colloidal bismuth subcitrate, Intrachol, Repliderm, 5FU (5-fluorouracil), and other indications of Cystadane. In December 1998, the Company sold its rights to colloidal bismuth subcitrate for $750,000, and is evaluating the potential value of its rights to one or more of the other proposed products that were discontinued in 1997. Depending on the availability of financing in subsequent periods, the Company may continue development of one or more of the proposed products that have been discontinued based on the criteria described under the Product Acquisition section. In addition, prior to 1997, the Company discontinued development activities on two proposed products, which proved to have little potential future value.

PRODUCTS

The following tables summarize certain information relating to the Company's products:

MARKETED PRODUCTS

APPROVED PRODUCT	APPLICATION	NDA APPROVAL DATE	U.S. PATENT ISSUED OR APPLIED FOR	ORPHAN DRUG STATUS **
Antizol(R) (fomepizole) Injection (Antidotes)	Antidote for ethylene glycol (antifreeze) or suspected ethylene glycol ingestion in humans	December 1997	No	Granted
	Antidote for methanol or suspected methanol ingestion in humans	December 2000	No	Granted
Busulfex(R) (busulfan) Injection (Oncology Support)	For use in combination with cyclophosphamide as a conditioning regimen prior to allogenic hematopoietic cell transplantation for chronic myelogenous leukemia ("CML")	February 1999	Yes	Granted
Elliotts B(R) Solution (buffered intrathecal electrolyte/ dextrose solution)	Diluent for intrathecally administered methotrexate sodium and cyarabine	September 1996	No	Granted
Cystadane(R) (betaine anhydrous for oral solution)	Homocystinuria, a genetic disease	October 1996	No	Granted
Antizol-Vet(R) (fomepizole) for Injection	Antidote for ethylene glycol (antifreeze) or suspected ethylene glycol ingestion in dogs	November 1996	No	Five year period of exclusivity
Sucraid(R) (sacrosidase) oral solution	Sucrase deficiency, a genetic disease	April 1998	No	Granted

PRODUCTS UNDER DEVELOPMENT

INVESTIGATIONAL PRODUCTS	PROPOSED APPLICATION	PHASE OF DEVELOPMENT *	U.S. PATENT ISSUED OR APPLIED FOR	ORPHAN D DESIGNAT **
Xyrem(R) (sodium oxybate) oral Solution (Sleep Disorders)	Under investigation for the treatment of narcolepsy	(1)	Yes	Yes

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* Development Phases are discussed under "Business -- The Regulatory Process".

** Orphan Drug Designation and Status are discussed under "Business -- Proprietary Rights"

(1) New Drug Application submitted October 2, 2000, approvable letter issued July 2, 2001.

APPROVED PRODUCTS

ANTIZOL (FOMEPIZOLE) INJECTION

Antizol received marketing clearance from the FDA in December 1997 for suspected or confirmed ethylene glycol poisonings and December 2000 for suspected or confirmed methanol poisonings. The Company commenced shipping Antizol in December 1997. Antizol is primarily used in a hospital setting and is distributed for the Company by an affiliate of Cardinal Health. When ingested by humans, ethylene glycol (found in antifreeze) and methanol (found in windshield wiper fluid) can lead to death or permanent and serious physical damage. In a survey conducted in 1999 by the American Association of Poison Control Centers, over 6,300 cases of ethylene glycol poisoning were reported to United States poison control centers. In the same year, there were approximately 600 treatments for such poisonings. The Company believes that hospital pharmacies will continue to stock Antizol because it is important to treat poisoned patients very quickly in order to improve the chances of successful recovery. For 2001, Antizol contributed approximately 43% of the Company's total revenues. The Company estimates that 30% of the 5,100 hospitals with emergency rooms currently stock the product. The Company expects to see limited incremental stocking by hospitals in 2002. Future sales will be based more on usage as stocking levels are expected to plateau. The Company has also received marketing approval for Antizol in Canada for the treatment of suspected or confirmed ethylene glycol poisonings.

The Company has obtained orphan drug status for Antizol as an antidote to treat ethylene glycol and methanol poisonings, which provides marketing exclusivity to the Company through December 2004 for ethylene glycol and December 2007 for methanol. Fomepizole, the active ingredient in Antizol, is a known compound and is not patentable. The Company has contracted with a third party for the production of Antizol under GMP conditions. The Company, through a sublicense agreement with Mericon Investment Group, Inc. ("MIG"), has an exclusive, worldwide license to develop and market Antizol, which expires in

July 2013, subject to a five year renewal through July 2018 exercisable by MIG at the request of the Company.

BUSULFEX (BUSULFAN) INJECTION

Following an accelerated six-month priority review, the Company received regulatory approval from the FDA in February 1999 to market Busulfex in the United States. The FDA approved Busulfex for use in combination with cyclophosphamide as a conditioning regimen prior to allogeneic hematopoietic progenitor cell transplantation for chronic myelogenous leukemia ("CML"). The first commercial sales of Busulfex occurred in February 1999, within two weeks of FDA approval. Busulfex also received marketing approval in Canada in September 1999, in Israel in February 2000 and in South Korea in December 2001. The indications approved in Canada, Israel and South Korea provide for use of Busulfex as a conditioning regimen prior to bone marrow or hematopoietic progenitor cell transplantation, when used in combination with other chemotherapeutic agents and/or radiotherapy. Included in the Canadian indication are acute lymphocytic leukemia, non-lymphocytic leukemia, acute myeloid leukemia, non-Hodgkin's lymphoma, Hodgkin's disease, multiple myeloma, myelodysplastic syndrome, breast cancer, ovarian cancer and several genetic diseases.

In December 2000, the Company signed a definitive agreement granting Kirin Brewery Co., LTD exclusive rights to market and distribute Busulfex in Asia. Kirin is a diversified company with annual sales of more than $14 billion worldwide in the food and beverage industry. Kirin expanded into the pharmaceutical industry in 1982 and has established a strong presence in the fields of nephrology, cancer and cell production, immunology and allergy. The Company began shipments of clinical trial materials to Kirin in 2001.

In October 1999, the Company signed a definitive agreement with Pierre Fabre Medicament, granting the French company exclusive rights to market and distribute Busulfex in 21 European countries, Argentina, and South Africa. Pierre Fabre will market Busulfex to transplant centers through 75 oncology marketing and sales personnel. The Company signed an agreement with IDIS World Medicine in December 1999 to distribute Busulfex on a named patient basis to requesting physicians outside of the United States, Canada and Israel. The Company began shipments to IDIS in January 2000. An NDA was submitted in Europe in late 2001. The Company does not expect approval until late 2002 or early 2003 at the earliest.

Bone marrow and peripheral stem cell transplants are collectively known as hematopoietic progenitor cell transplants, but are more commonly referred to as stem cell or bone marrow transplants. This year approximately 20,000 stem cell transplants are expected to be performed in the United States, with another 20,000 expected to be performed outside the United States. One of the many complex steps in performing a stem cell transplant includes using high doses of chemotherapeutic drugs, such as Busulfex, and/or radiation as a "conditioning regimen" to destroy the abnormal cancer cells in the bone marrow and to create "space" for the engraftment of transplanted stem cells. The stem cell transplant then replaces the diseased or damaged marrow to grow into healthy marrow. Of the 20,000 procedures expected to be performed in the United States, more than 4,000 of these may receive some form of busulfan, as part of the pre-transplantation conditioning regimen. The Company is marketing Busulfex to hematologists and oncologists who perform stem cell transplants at cancer treatment centers.

Adoption of Busulfex into standard busulfan-based regimens has been steady, and several clinicians in leading institutions have initiated new research protocols using Busulfex to enhance the conditioning regimen, based on clinical

data and experience with Busulfex. Several leading United States research centers have indicated to the Company that they have initiated or intend to initiate new study protocols specifying the use of Busulfex.

Many other drugs are currently being developed for use in the BMT area. Although most of these new drugs are used for a different purpose during the bone marrow transplant and not in the conditioning regimen, some of them may compete with Busulfex. The Company is aware that SuperGen Inc., which acquired Sparta Pharmaceutical in 1999, now owns rights to another form of intravenous busulfan which was in development by Sparta prior to its acquisition. SuperGen holds orphan drug designation for its intravenous busulfan and could seek orphan drug status for a similar or different indication, if SuperGen were to proceed with development of its intravenous formulation and if the FDA approves an NDA for such a product.

The Company has obtained orphan drug status for Busulfex for the approved indication, which provides marketing exclusivity for that indication to the Company through February 2006. The Company has contracted with a third party for the production of Busulfex under GMP conditions. In addition, the Company has an exclusive, worldwide license except for Australia from M.D. Anderson Cancer Center, The University of Texas, and The University of Houston (collectively, the "busulfan licensors") to develop and market this intravenous form of busulfan, which is effective for the term of busulfan licensors' patent rights. The busulfan licensors have been granted two U.S. patents covering the licensed product's formulation and its use in bone marrow transplants and other conditions, which expire in September 2016 and July 2015, respectively. The product is distributed for the Company by an affiliate of Cardinal Health

ELLIOTTS B SOLUTION (BUFFERED INTRATHECAL ELECTROLYTE/DEXTROSE INJECTION)

Elliotts B Solution received marketing clearance from the FDA in September 1996. The first commercial sales of Elliotts B Solution occurred in December 1996. Elliotts B Solution is primarily used in a hospital setting and is distributed for the Company by an affiliate of Cardinal Health. Elliotts B Solution is a buffered diluent for the intrathecal administration (injection into the fluid space surrounding the central nervous

system) of chemotherapeutic agents. Intrathecal injections are most commonly made in treating acute lymphoblastic leukemia ("ALL"). ALL is the most prevalent form of leukemia in children. Due to advances in chemotherapy, the cure rate for ALL has improved dramatically in the past 30 years, going from almost zero to nearly 75% today. As a part of modern chemotherapy, doctors often administer a series of up to 20 injections of methotrexate sodium into the cerebrospinal fluid of patients. Elliotts B Solution is comparable in pH, electrolyte composition, glucose content and osmolarity to cerebrospinal fluid. It is estimated that cerebrospinal injections of methotrexate sodium are administered to about 6,000 people in the United States on an annual basis. Elliotts B Solution revenues have not been material nor does the Company expect that revenues to be material to the Company's financial results.

The Company has obtained orphan drug status for the use of Elliotts B Solution as a diluent for methotrexate sodium or cytarabine, which provides marketing exclusivity through September 2003. Elliotts B Solution is a known compound that has been used previously for the intrathecal administration of chemotherapeutic agents and is, therefore, not patentable. The Company has contracted with a third party for the production of Elliotts B Solution under GMP conditions. No license was required for the Company to develop and market Elliotts B Solution.

OTHER APPROVED PRODUCTS

CYSTADANE (BETAINE ANHYDROUS FOR ORAL SOLUTION)

Cystadane received marketing clearance from the FDA in October 1996. The first commercial sales of Cystadane occurred in December 1996. Cystadane is principally distributed on a non-exclusive basis by Chronimed Inc. directly to patients in the United States through its mail order pharmacy. The Company believes that the small size of the market and the high medical value of Cystadane justify the limited resources required by the Company to continue making this product available to patients. It is the first agent approved by the FDA for the treatment of homocystinuria, an inherited metabolic disease. The clinical consequences are wide-ranging and include dislocation of the ocular lens, early (under age 30) thromboembolism, developmental and mental retardation and reduced life span related to elevated plasma homocysteine levels. It has been estimated that homocystinuria occurs about once in every 200,000 live births worldwide. There are estimated to be 1,000 patients with homocystinuria in the United States. The annual market potential for Cystadane is approximately $500,000 in the United States. The Company does have some sales revenue generated outside of the United States. Cystadane revenues met the Company's expectations in 2001 and are expected to grow slightly in subsequent periods.

The Company has obtained orphan drug status for Cystadane for the treatment of homocystinuria, which provides marketing exclusivity to the Company through October 2003. Betaine anhydrous, the active ingredient in Cystadane, is a known compound and is not patentable. The Company has contracted with a third party for the production of Cystadane under GMP conditions. No license was required for the Company to develop and market Cystadane.

The Company is not currently sponsoring any clinical trials with/for Cystadane. The Company is aware, however, of clinical trials being conducted by independent investigators to assess the safety and efficacy of Cystadane as a stand alone or adjunctive therapy for the following indications: Non-alcoholic steatohepatitis, Rett syndrome, rheumatoid arthritis and hyperhomocystinemia. The Company does not expect that the results of any of these clinical trials to significantly enhance or decrease the current limited market potential for Cystadane on the near future.

ANTIZOL-VET (FOMEPIZOLE) FOR INJECTION

In November 1996, the Center for Veterinary Medicine of the FDA approved Antizol-Vet as a treatment for dogs that have ingested or are suspected of having ingested ethylene glycol. The first commercial sales of Antizol-Vet occurred in January 1997. It is estimated that at least 10,000 cases of ethylene glycol poisoning occur in dogs each year. The earlier an ethylene glycol poisoned dog is treated with Antizol-Vet, the more likely that there will be a positive outcome. The annual market potential for Antizol-Vet is expected to be under $300,000. The Company has found that stocking of this product has been limited due to its high cost,

but it is ordered when a poisoning occurs. Antizol-Vet revenues met the Company's expectations in 2001 and are expected to remain constant or decline in subsequent periods.

Federal law provided the Company with a marketing exclusivity period through November 2001 for the use of Antizol-Vet in dogs for the approved indication. Fomepizole, the active ingredient in Antizol, is a known compound and is not patentable. The Company has contracted with a third party for the production of Antizol-Vet under GMP conditions.

The Company has partnered with several leading regional and national

veterinary wholesalers to distribute Antizol-Vet to veterinary clinics. It is believed that the current partners effectively and efficiently encompass the entire country with limited sales territory overlap, thus helping prevent downward retail pricing pressures. The Company does not anticipate adding additional distribution partners.

SUCRAID (SACROSIDASE) ORAL SOLUTION

Sucraid received marketing clearance from the FDA in April 1998. The first commercial sales of Sucraid occurred in July 1998. Sucraid is principally distributed by an affiliate of Cardinal Health directly to patients in the United States. The FDA approved Sucraid to be used for oral replacement therapy of genetically determined sucrase deficiency, which is part of congenital sucrase isomaltase deficiency (CSID). Sucraid is used as a replacement for an enzyme in the small intestine that is necessary for the digestion of sucrose, which is common table sugar. The Company believes that the small size of the market and the high medical value of Sucraid justify the limited resources required by the Company for making this product available to patients. The annual market potential for Sucraid is expected to be approximately $500,000 in the United States. Sucraid revenues met the Company's expectations and are expected to grow slightly in 2002.

The primary symptoms of CSID include severe watery diarrhea, chronic malabsorption and, in infants and toddlers, failure to thrive. Other common symptoms include nausea, vomiting, abdominal cramps and abdominal pain following the consumption of foods containing sucrose. Prior to the approval of Sucraid, the only specific treatment of CSID available to patients was the life-long adherence to a sucrose-free diet. Compliance with a sucrose-free diet is very difficult because sucrose is found in many foods in the typical American diet. Nonspecific symptomatic treatments include antidiarrheal, antispasmodic and antiflatulence drugs, all of which are limited in their efficacy. Sucraid is a specific replacement of the missing enzyme responsible for CSID.

Sacrosidase, the active ingredient in Sucraid, is a known compound and is not patentable. The Company has obtained orphan drug status for Sucraid for the approved indication, which provides marketing exclusivity to the Company through April 2005. The Company has contracted with a third party for the production of Sucraid under GMP conditions. In addition, the Company has an exclusive, worldwide license from Hartford Hospital to develop and market Sucraid, which expires in April 2005. The license provides for two five year extension options, the first through April 2010 and the second through April 2015, unless either party decides to terminate the license within 90 days prior to April 2005 or April 2010.

SLEEP DISORDERS -- INVESTIGATIONAL PRODUCT

XYREM (SODIUM OXYBATE) ORAL SOLUTION

Narcolepsy is a chronic neurologic sleep disorder in which sleep is "fragmented", that is, does not occur in an integrated and cohesive manner. This fragmentation results in excessive daytime sleepiness, unavoidable daytime sleep attacks, cataplexy or sudden loss of muscle control provoked by emotions, sleep paralysis or brief periods of muscle paralysis and hallucinations, or vivid and sometimes frightening dreaming when falling asleep or waking up. Other related symptoms include broken night-time sleep, disturbances of auditory and visual perception, and lapses of consciousness and memory problems. These symptoms can lead to a variety of complications, such as limitations on education and employment opportunities, driving or machine accidents, difficulties at work resulting in disability, forced retirement or job dismissal, and depression. Narcolepsy is thought to affect 140,000 to 180,000 persons in the United States. Approximately 75,000 of these patients are thought to be diagnosed. Narcolepsy patients usually begin to develop symptoms between the ages of 15-25,

with a smaller number developing symptoms between the ages of 35-45. Symptoms have been reported in patients as young as five years of age.

The usual treatment for narcolepsy includes symptomatic treatment of excessive daytime sleepiness and sleep attacks with stimulants or wakefulness promoting agents. The symptoms of cataplexy, sleep paralysis and hypnagogic hallucinations are typically treated with tricyclic antidepressants ("TCAs") or selective serotonin reuptake inhibitors ("SSRIs"). These treatment regimens, in addition to limited efficacy, are often unsatisfactory for a number of other reasons. Amphetamines and other stimulants often cause undesirable side effects such as insomnia, hypertension, palpitations, irritability and, at higher doses, may mimic the symptoms of schizophrenia. Patients often build tolerance to the TCAs and SSRIs and doses are increased to obtain clinical effectiveness at high doses. These medications can cause the side effects of dry mouth, impotence, loss of libido, and increased heart rate. Clinical results with Xyrem suggest that it is effective in the treatment of narcolepsy symptoms. Administered at night, it is believed to consolidate sleep and has been shown to reduce cataplexy attacks, and to reduce the severity of daytime sleepiness when used in combination with stimulants during the day. More than 300 narcolepsy patients have been exposed to clinical doses with an acceptable side effect profile. Xyrem does not appear to have the side effects associated with TCAs and SSRIs. Narcoleptic patients could be treated with Xyrem at night and, if needed, with stimulants during waking hours.

During 1998, the Company completed a clinical trial with over 130 patients at 18 sleep centers, which assessed Xyrem at different dose levels against placebo. When compared against placebo, higher doses of Xyrem demonstrated a reduction in the number and severity of cataplexy attacks per week, the primary measure of efficacy in this clinical trial. The secondary measure of efficacy was the reduction of excessive daytime sleepiness, as measured by the Epworth Sleepiness Scale, a subjective, but validated measure of EDS. At higher doses, Xyrem appeared to reduce EDS beyond the improvement provided by stimulants which patients continued to take during the trial.

During the first quarter of 1999, the Company commenced a Treatment IND program, which is a clinical trial, to collect additional clinical safety data necessary for the submission of an NDA. The FDA permits an investigational drug, like Xyrem, to be used under a Treatment IND if there is sufficient evidence of safety and effectiveness and the drug is intended to treat a serious disease. The FDA also authorized the Company to seek reimbursement from patients and their insurance providers for the use of Xyrem in this trial, which has allowed the education of certain third party payors regarding Xyrem.

Gamma hydroxybutyrate (GHB) is the active ingredient in Xyrem. Illicitly produced GHB has been reported to be a drug of abuse. On February 18, 2000, the President signed PL 106-172, a public law which makes GHB a Schedule I substance. Schedule I is the designation by which illegal drugs are controlled. The bill further delineates GHB products being studied under Food and Drug Administration (FDA) approved protocols or approved for commercial sale as Schedule III substances.

Each state has the ability to schedule products more strictly or equivalent to the Federally designated schedule. Most states have adopted, either administratively or legislatively, the I/III schedule as described above. The Company continues its efforts to ensure consistency across all states by the time of commercial launch.

Sodium oxybate (GHB), the active ingredient in Xyrem, is a known compound

and is not patentable. The Company has received an orphan drug designation for its proposed use of Xyrem. No license was required for the Company to develop Xyrem nor will a license be required to market Xyrem, if approved by the FDA. The Company has contracted with third party bulk drug and drug product manufacturers for the production of Xyrem.

The Company submitted its NDA for Xyrem on October 2, 2000. The NDA was granted priority review status by the FDA, meaning the FDA had a goal of reaching a decision to either approve or disapprove or delay a decision regarding the NDA within 180 days of submission. On March 2, 2001, the FDA granted a 90 day extension to the Company's NDA in order that the Company could submit additional data requested by the FDA. On June 6, 2001, the FDA held a meeting of the Peripheral and Central Nervous System Advisory Committee to consider Xyrem. The Advisory Committee voted affirmatively that Xyrem was

effective in treating cataplexy, but was split on the issue of Xyrem's safety due to the relatively small size of the safety database. As a result, the FDA issued an Approvable Letter for Xyrem on July 2, 2001. The Approvable Letter defined issues that required resolution before approval could be granted for the treatment of cataplexy. On October 23, 2001, the Company announced that the FDA had accepted the Company's response, in the form of an amendment, to the Approvable Letter. The amendment represented the Company's complete response to the issues contained in the FDA's Approvable Letter. The amendment includes revisions to product labeling and the risk management program, a safety update of ongoing clinical trials, and respiratory data collected during all night polysomnographic recordings of narcolepsy patients in a clinical trial completed in late 2000. The Company believes it has addressed all questions and issues the FDA may have regarding the NDA for Xyrem. The FDA assigned April 9, 2002 as its action deadline for this NDA.

The Company is conducting a Phase III (b) third clinical trial for Xyrem, which the FDA has previously indicated is not required for the Company's NDA submission. This controlled clinical trial assesses the efficacy of Xyrem for treating excessive daytime sleepiness (EDS) related to narcolepsy as its primary endpoint. This trial is expected to be completed in late 2002.

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 investors should carefully consider the following information about these risks before buying shares of common stock.

WE HAVE A HISTORY OF LOSSES.

We have been unprofitable since our inception in 1994. We expect operating losses in 2002 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 ("FDA") approved products. We cannot assure you that we will ever generate sufficient product revenues to achieve profitability.

THERE ARE RESTRICTIONS ON OUR ABILITY TO RAISE ADDITIONAL CAPITAL. IF WE ARE UNABLE TO OBTAIN ADDITIONAL FINANCING, WE MAY NOT BE ABLE TO SUPPORT OUR CURRENT OR FUTURE BUSINESS OPERATIONS.

On July 23, 1998, we completed the private sale to UBS Capital II, LLC of $7.5 million of Senior Convertible Preferred Stock. On August 2, 1999, we completed another private sale to UBS Capital II of $3.0 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. On December 7, 2001, we completed the private sale of 1.7 million shares of common stock to a group of investors led by Alta BioPharma Partners II, L.P. In connection with this sale, UBS Capital II agreed to forfeit its right as a preferred stockholder to enforce the restrictions and covenants relating to our ability to incur additional indebtedness and issue additional equity securities. However, we are still subject to other restrictions and covenants relating to the preferred stock, and these restrictions could make it more difficult and more costly for us to obtain additional capital.

We expect our spending for research and development and sales and marketing to increase significantly in fiscal 2002. Although we believe that we have sufficient capital to meet our business objectives in fiscal 2002, if we expand our business plan, or unanticipated events occur, we may need additional capital. We cannot assure you that additional sources of capital will be available to us, or if available, on terms acceptable to us. If we issue additional equity securities, your ownership interest may be diluted.

THE MARKET PRICE OF OUR COMMON STOCK COULD FLUCTUATE IN RESPONSE TO QUARTERLY OPERATING RESULTS AND OTHER FACTORS.

The market price of our common stock could fluctuate significantly in response to a number of factors, including:

- our quarterly financial performance;
- 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; and
- small float or number of shares of our common stock available for sale and trade.

The market value and 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) net tangible assets in excess of $4.0 million as reported on Form 10-Q or Form 10-K or (2) a market capitalization of at least $50.0 million. Net tangible assets are defined as total assets less the sum of total liabilities and intangible assets. Market capitalization is defined as total outstanding shares multiplied by the last sales price quoted by Nasdaq. Although we currently meet the requirements for listing on the Nasdaq National Market, we cannot assure you that we will continue to meet these requirements. The Nasdaq National Market has issued new listing qualifications which will become effective November 2002, and which will replace the net tangible asset requirement with a minimum net equity requirements of $10.0 million. At September 30, 2001, we met the new listing qualifications with respect to market

capitalization. We cannot assure you that we will continue to meet the new listing qualification requirements.

The market price of our common stock may also fluctuate significantly in response to other factors over which we have no control and that may not be directly related to us. Fluctuations or decreases in the trading price of our common stock may adversely affect your ability to trade your shares and you may lose all or a part of your investment. In addition, fluctuations and decreases in our stock price could adversely impact our business and our ability to raise capital through additional equity financings.

THERE IS A LIMITED MARKET FOR OUR PRODUCTS.

While we will seek to obtain and market products that address diseases that affect patient populations larger than those affected by orphan diseases (200,000 or fewer patients in the United States), many of our opportunities will address orphan diseases. 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. We cannot assure 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 RELY ON THE LIMITED PROTECTION OF THE ORPHAN DRUG ACT.

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 a "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 obtaining "orphan drug designation" for a product from the FDA. 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, nor does it provide any advantage in, or shorten the duration of, the regulatory approval process.

The second step in obtaining limited protection under the Orphan Drug Act for a specific product is acquiring the FDA's recognition of "orphan drug status." This step involves submission of a New Drug Application ("NDA") to the FDA containing all clinical study results, safety and manufacturing information and requesting approval to market a drug for the designated indication. The FDA will grant orphan drug status to the first company to receive approval of an NDA for the designated indication. Orphan drug status gives a company the exclusive right to market the approved product in the United States for a period of seven years, subject to certain limitations. Obtaining orphan drug status for a particular product may not, however, prevent another company from developing or marketing the same drug having a different formation or composition for the same or different indication. In addition, orphan drug status does not provide any marketing exclusivity in foreign markets. While 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 or will have meaningful or material value to us. Although certain foreign countries provide exclusivity, development and marketing benefits for orphan drugs, we cannot assure you that such benefits can be

obtained or, if obtained, will be of material value to us.

We have obtained orphan drug status for Antizol, Elliotts B Solution, Cystadane, Sucraid, and Busulfex. We have obtained orphan drug designation for Xyrem, our narcolepsy drug and our NDA requesting orphan drug status for Xyrem is currently under review by the FDA. If the FDA approves another company's NDA for sodium oxybate (the generic identity of the therapeutic agent for Xyrem) for the same indication as Xyrem prior to approving our NDA for Xyrem, that company will be entitled to exclusive marketing rights for sodium oxybate, and the FDA would not approve our application to market Xyrem for seven years, if at all. We are aware that the FDA has granted Teva (formerly Biocraft) orphan drug designation for the use of sodium oxybate to treat the symptoms of narcolepsy, however, we have obtained the exclusive right to use Teva's data for one controlled study included in our NDA submission. While we are not aware of any activities to develop sodium oxybate by any other U.S. company, we cannot assure you that such activities are not being conducted, or that the FDA will approve our NDA for Xyrem first for the designated indication. We also cannot assure you that the FDA will not grant orphan drug designation and orphan drug status to other competing products before or after approving our NDA for Xyrem.

Even if the FDA approves an NDA for a drug with an 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. We are aware that the FDA has granted Sparta Pharmaceutical, which was acquired by SuperGen Inc., orphan drug designation for an intravenous busulfan with an indication closely related to the indication for our product Busulfex. If the FDA approves an NDA for SuperGen's product for a different indication, SuperGen could seek orphan drug status for that product, which competes with Busulfex. In addition, the FDA does not restrict doctors from prescribing an approved drug for uses not approved by the FDA. 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 the 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

The European orphan drug act provides for up to ten years of market exclusivity for a pharmaceutical product that meets the requirement of the European orphan drug act. For a pharmaceutical product to qualify under the act, the prevalence (or incidence), of the condition being treated must not exceed five patients per 10,000 population. Our European partners have submitted and obtained orphan drug designation under the act for Busulfex and Cystadane, and in May 2001 we were granted orphan drug designation under the act for Antizol for use in methanol poisonings. While these products are currently designated as orphan drugs, we cannot assure you that these products will continue to qualify for orphan drug protection in Europe or that we will be able to obtain orphan drug protection in Europe for other or future products. We also cannot provide you any assurance that another company will not obtain an approval which would

block us from marketing our products in Europe.

THE SALE OF OUR PRODUCTS IS DEPENDENT UPON GOVERNMENTAL APPROVAL.

Government regulation in the United States and abroad is a significant factor in the testing, production and marketing of our products. Each product must undergo an extensive regulatory review process conducted by FDA and by comparable agencies in other countries. We cannot market any pharmaceutical product we develop or license as a prescription product in any jurisdiction, including foreign countries, without 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 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 in a timely manner, if at all, any product 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 for further studies to provide additional data on safety or effectiveness.

GOVERNMENTAL APPROVAL OF OUR PRODUCTS DOES NOT GUARANTEE FINANCIAL SUCCESS.

Six of our products have been approved for marketing by regulatory authorities in the United States or elsewhere. Even if we obtain FDA approval to market Xyrem, we cannot assure you that Xyrem or our other products will be commercially successful or achieve the expected financial results. 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. 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, primarily to focus our development efforts and resources on those products that fit within our three selected strategic therapeutic market segments: Antidote; Oncology Support; and Sleep Disorders or for which we believe there is an opportunity for growth or profitability. We cannot assure you that any of these discontinued products currently, or may in the future, have any value. Depending on available financing, we may develop one or more of these discontinued products in the future. We cannot assure you that we will

continue development of our current or any proposed products, or that we will

continue marketing all of our FDA approved products.

SIGNIFICANT GOVERNMENT REGULATION CONTINUES ONCE A PRODUCT IS APPROVED FOR SALE.

After the FDA's Division of Drug Marketing approves a drug, the FDA's Advertising and Communication division must accept the drug's marketing claims, which are the basis for the drug's labeling, advertising and promotion. We cannot assure you that the FDA will approve our proposed marketing claims. Failure to obtain approval of our proposed marketing claims could have a material adverse effect on our business and prospects.

The FDA requires that we conduct "post-marketing adverse event surveillance programs" to monitor any side effects that occur after any of our drug products are approved for marketing. If the surveillance program indicates unsafe side effects, the FDA may recall the product, and suspend or terminate our 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 withdrawal 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. To date, none of our products have been subject to an FDA recall. We cannot assure you that our products will not be subject to an FDA recall in the future.

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

WE DEPEND ON OTHERS FOR PRODUCT DEVELOPMENT OPPORTUNITIES.

We engage only in limited research to identify new pharmaceutical compounds. To build our product portfolio, we utilize a license and acquisition strategy. This strategy for growth requires us to identify and acquire pharmaceutical products targeted at niche markets within selected strategic therapeutic market segments. 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 or will be successfully developed, approved or marketed. We 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 license or acquire new pharmaceutical products, or to promote and market products successfully, would 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:

- 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 will meet, or continue to meet, the requirements specified in our current or any future license agreements. We cannot assure you that if any agreement is terminated, we will be able to enter into a similar agreement on terms as favorable as those contained in our existing license agreement.

WE DEPEND ON OTHERS TO MANUFACTURE AND SUPPLY THE PRODUCTS WE MARKET.

We do not have, and do not intend to establish, any internal product testing, synthesis of bulk drug substance, or manufacturing capability for drug product. Accordingly, we depend on others to supply and manufacture the components incorporated into all of our finished products. The inability to secure contracts for these components on acceptable terms could adversely affect our ability to develop and market our products.

Failure by parties with whom we contract to adequately perform their responsibilities may delay our 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 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 drug supply or manufacturing process to a new supplier or manufacturer. We believe that it could take as long as one year 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 undertaken to certify and maintain secondary sources of supply for drug substances or backup drug manufacturers for some products. If we lose either a supplier or a product manufacturer, we could run out of salable product to meet market demands or investigational product for use in clinical trials while we locate and then wait for FDA approval of a new drug supplier or manufacturer. We cannot assure you that any change in drug supplier or manufacturer or the transfer of a drug manufacturing processes to another third party would be approved by the FDA, or approved in a timely manner. The loss of, or change in, drug supplier or a drug manufacturer could have a material adverse effect on our business and prospects.

BULK DRUG SUPPLY

Bulk drug substance is the active chemical compound used in the manufacture of our drug products. We depend substantially on Ash Stevens, Inc. for the supply of bulk drug substance used in Busulfex, Antizol, and Antizol-Vet. If we were to lose Ash Stevens as a supplier, we would be required to identify a new supplier for the bulk drug substance used in products that provided approximately 88% of our total revenues in 2000 and 90% of our total revenues in 1999, and which are expected to account for approximately 85% of our revenues in 2001. We depend substantially on Lonza, Inc. for the supply of bulk drug substance used in Xyrem. If we were to lose Lonza as a supplier, we would be required to identify a new supplier before an NDA is submitted for Xyrem. We also cannot assure you that our bulk drug supply arrangements with Ash Stevens and Lonza, or any other future such supplier, might not change in the future. We cannot assure you that any change would not adversely affect production of Busulfex, Antizol, Antizol-Vet, Xyrem, or any other drug the Company might attempt to develop or market.

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 depend substantially on an affiliate of Boehringer Ingelheim for drug product manufacturing of Busulfex, Antizol, and Antizol-Vet. Upon FDA approval of Xyrem, an affiliate of DSM, N.V. has been authorized to manufacture Xyrem. If we were to lose Boehringer as a manufacturer, we would be required to identify a new manufacturer for drug products that provided approximately 88% of our total revenues in 2000 and 90% of our total revenues in 1999, and which are expected to account for approximately 85% of our total revenues in 2001. We cannot assure you that our drug product manufacturing arrangements with Boehringer and DSM, N.V. will not change or that the manufacturing services will continue to be available on terms satisfactory to us. Any change in our manufacturing agreements with Boehringer and DSM, N.V could adversely affect production of Busulfex, Antizol, Antizol-Vet or Xyrem, or any other drug that we might attempt to develop or market, which could have a material adverse effect on our business and prospects.

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 prescriptions. Failure by our contractors to comply with FDA or DEA requirements or applicable foreign requirements could significantly delay our ability to commercialize or continue to market our products. Either result could have a material adverse effect on our business and prospects. Our contractors failure to comply with good marketing practices or other legal requirements could also result in seizure of violative products, injunctive actions brought by the federal government or criminal and civil liability for Orphan, our officers, or our employees. We cannot assure you that we will be able to maintain relationships either domestically or abroad with contractors whose facilities and procedures comply with, or will continue to comply with, FDA or DEA requirements or applicable foreign requirements.

WE DEPEND UPON OTHERS FOR DISTRIBUTION OF OUR PRODUCTS.

We have an agreement with CORD Logistics, Inc., a subsidiary of Cardinal Health, Inc., to provide integrated distribution and operations services to support transactions between us and our wholesalers, specialty distributors, and direct customers. CORD also provides reimbursement management, patient assistance and information hotline services and specialty distribution and marketing services to physician practices with respect to our products. CORD currently distributes Busulfex, Cystadane, Elliotts B Solution, Antizol, Antizol-Vet, and Sucraid. CORD may also distribute future products should those products receive marketing clearance from the FDA. We are substantially dependent on CORD's ability to successfully distribute Busulfex, Elliotts B Solution, Antizol, Antizol-Vet, and Sucraid and other potential products.

Chronimed Inc. is the principal distributor, on a non-exclusive basis, in the United States for Cystadane. Chronimed distributes this product directly to patients through its mail order pharmacy. We are substantially dependent on Chronimed's ability to successfully distribute Cystadane directly to patients in the United States.

We cannot assure you that our distribution arrangements with CORD, Chronimed 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.

WE DEPEND ON FOREIGN COMPANIES TO SELL OUR PRODUCTS OUTSIDE OF THE UNITED STATES AND OUR INABILITY TO ESTABLISH AND MAINTAIN MARKETING ALLIANCES WITH FOREIGN COMPANIES COULD ADVERSELY AFFECT OUR BUSINESS.

Our strategy to sell our products outside of the United States is to license foreign marketing and distribution rights to a foreign company after a NDA is submitted to, or approved by, the FDA in the United States. We consider Europe, Asia and Canada our most attractive foreign markets. Our current foreign developments are:

- Europe. We have licensed the marketing and distribution rights for Busulfex, Antizol, Cystadane and Sucraid in Europe. If our licensees' registration and distribution efforts are not successful, it may be difficult for us to contract with other distributors in Europe for these products. 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" or "emergency use." Emergency use distribution of our products is expected to result in limited revenues for us.

- Asia. We have licensed marketing and distribution rights for Busulfex in Japan, the Peoples Republic of China, Taiwan and South Korea. Use and distribution of all products in these countries, except South Korea, is limited to clinical trials until full regulatory approval is obtained. Revenues prior to full approval are not expected to be material. Full regulatory approval for marketing of these products in

South Korea was obtained in late 2001. We do not expect to generate material revenues from our South Korean marketing and distribution activities.

- Canada. We have licensed marketing and distribution rights for Antizol. We do not expect to generate material revenues from these marketing and distribution activities.

- Australia and New Zealand. We have licensed marketing and distribution rights for Cystadane and Sucraid in Australia and New Zealand. We do not expect to generate material revenues from these marketing and distribution activities.

- Central America. We have licensed marketing and distribution rights for Elliotts B Solution in Central America. We do not expect to generate material revenues from these marketing and distribution activities.

- Israel. We have licensed marketing and distribution rights for Antizol, Busulfex, Cystadane, Elliotts B Solution and Sucraid in Israel. Full regulatory approval for all products except Antizol was obtained in February 2000. Antizol has been submitted for approval. We do not expect to generate material revenues from these marketing and distribution activities.

- Turkey. We have licensed marketing and distribution rights for Busulfex

in Turkey. We do not expect to generate material revenues from these marketing and distribution activities.

We depend on our foreign licensees for the regulatory registration of our products in foreign countries. We cannot assure you that our licensees will obtain such registration. In addition, we cannot assure you 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 our foreign licensees will be successful in marketing and selling our products in their respective territories.

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, 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, none of our products have been subject to an FDA recall. We cannot assure you that our products will not be subject to an FDA recall in the future.

THE PRICES WE CHARGE FOR OUR PRODUCTS ARE SUBJECT TO GOVERNMENTAL REGULATION WHICH COULD ADVERSELY AFFECT OUR ABILITY TO RECOVER OUR PRODUCT DEVELOPMENT COSTS AND OUR FINANCIAL PERFORMANCE.

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 cannot predict the level of pricing flexibility we will have with respect to our products or whether we, or users of our products, will be reimbursed for newly approved health care products.

In the United States, we expect continuing federal and state proposals to implement government control of the pricing and profitability of prescription pharmaceuticals. Cost controls could decrease, or limit, the price we receive for our current and future products. 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 and prospects. Furthermore, federal and state regulations govern or influence reimbursement of health care providers for medical treatment of certain patients. We cannot assure you that actions taken by federal 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 MAY BE UNABLE TO PROTECT OUR PROPRIETARY INFORMATION, WHICH COULD NEGATIVELY AFFECT OUR ABILITY TO COMPETE IN THE PHARMACEUTICAL INDUSTRY.

The pharmaceutical industry and the investment community place considerable importance and value on obtaining patent 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 patents issued to us 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 and proposed products, Cystadane, Elliotts B Solution, Antizol, Antizol-Vet, Xyrem and Sucraid, are in the public domain and are not currently subject to patent protection in the United States. However, we have filed a patent application with respect to our formulation of Xyrem oral solution. A United States patents issued to The University of Texas System and The University of Houston-University Park, the group from whom we license the formulation for Busulfex, covers our formulation and use of Busulfex. We could, however, incur substantial costs asserting any infringement claims that we may have against others.

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.

WE FACE INTENSE COMPETITION IN THE PHARMACEUTICAL 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 designation and 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.

IF WE ARE UNABLE TO RESPONSE TO RAPIDLY CHANGING TECHNOLOGIES AND OTHER DEVELOPMENTS, WE MAY NOT BE ABLE TO COMPETE EFFECTIVELY.

The pharmaceutical industry has experienced rapid and significant technological change as well as structural changes, such as those brought about by changes in heath 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 $20 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 2. PROPERTIES

The Company currently occupies approximately 15,000 square feet of leased office space located in Minnetonka, Minnesota at a monthly rent of approximately $25,000, including operating expenses. This lease expires on October 31, 2003.

ITEM 3. LEGAL PROCEEDINGS

None.

ITEM 4. SUBMISSION OF MATTERS TO VOTE OF SECURITY HOLDERS

None.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON STOCK AND RELATED STOCKHOLDER MATTERS

The Company's Common Stock trades on the National Market tier of The Nasdaq Stock Market under the symbol ORPH. The following table sets forth the quarterly high and low sales prices for the Company's Common Stock for the years ended December 31, 2001 and December 31, 2000.

	HIGH	LOW
YEAR ENDED DECEMBER 31, 2001		
January 1 through March 31	$18.375	$6.375
April 1, through June 30	$15.000	$9.500
July 1 through September 30	$12.200	$6.500
October 1 through December 31	$14.100	$7.240
YEAR ENDED DECEMBER 31, 2000		
January 1 through March 31	$19.250	$4.688
April 1 through June 30	$11.000	$5.750
July 1 through September 30	$13.000	$9.125
October 1 through December 31	$16.000	$9.625

As of March 20, 2002, the Company's Common Stock was held by 275 shareholders of record and the Company estimates that there were approximately 3,000 beneficial owners of its Common Stock on such date.

The Company has never declared or paid any dividends and does not anticipate paying dividends on its Common Stock in the foreseeable future. The Company currently intends to retain future earnings, if any, for use in the Company's business. The payment of any future dividends on its Common Stock will be determined by the Board of Directors in light of conditions then existing, including the Company's earnings, financial condition and requirements, restrictions in financing agreements, business conditions and other factors.

ITEM 6. SELECTED FINANCIAL DATA

The following selected financial data of the Company as of December 31, 2001 and 2000 and for the three years ended December 31, 2001, 2000 and 1999, are derived from, and are qualified by reference to, the financial statements of the Company audited by Ernst & Young LLP, independent auditors, included elsewhere in this Form 10-K. The selected financial data as of December 31, 1999, 1998 and 1997 and for each of the two years ending December 31, 1998 and 1997 are derived from financial statements which are not included herein. The information set forth below should be read in conjunction with "Management's Discussion and Analysis of Financial Condition and Results of Operations," the Financial Statements and Notes thereto and other financial information included elsewhere in this Form 10-K.

FINANCIAL POSITION

	DECEMBER 31,				
	2001	2000	1999	1998	1997

Cash, cash equivalents and available-for-sale securities	$19,011,245	$11,417,254	$ 4,032,914	$ 7,521,483	$ 7,169
Working capital	18,010,707	10,266,029	3,161,324	5,274,550	4,479
Total assets	22,346,276	15,296,885	6,241,178	9,046,730	8,238
Accumulated deficit	(54,073,165)	(47,178,667)	(40,243,874)	(34,433,640)	(26,196
Total shareholders' equity	18,394,207	10,742,927	3,561,208	5,575,577	3,645

FINANCIAL RESULTS

	FOR THE YEAR ENDED DECEMBER 31, 2001	FOR THE YEAR ENDED DECEMBER 31, 2000	FOR THE YEAR ENDED DECEMBER 31, 1999	FOR THE YEAR ENDED DECEMBER 31, 1998	FO YEA DECE
Revenues	$11,274,110	$11,185,634	$ 6,457,406	$ 5,048,308	$
Cost of sales	1,591,826	1,532,446	803,562	1,118,644	
Gross profit	9,682,284	9,653,188	5,653,844	3,929,664	
Operating expenses					
Research and development	4,933,278	6,832,130	4,975,706	6,611,011	6
Sales and marketing	6,259,045	5,606,506	3,430,539	2,739,299	1
General and administrative	4,807,847	4,094,905	2,756,827	2,994,341	2
Contract termination fee	--	--	--	--	2
Loss from operations	(6,317,886)	(6,880,353)	(5,509,228)	(8,414,987)	(11
Other income, net	321,315	793,238	287,989	177,885	
Net loss	(5,996,571)	(6,087,115)	(5,221,239)	(8,237,102)	(11
Less:					
Preferred stock dividend	903,053	872,024	682,872	249,658	
Net loss applicable to common shareholders	$(6,899,624)	$(6,959,139)	$(5,904,111)	$(8,486,760)	$(11
Basic and diluted loss per common share	$ (0.80)	$ (0.86)	$ (0.90)	$ (1.36)	$
Weighted average shares outstanding	8,597,331	8,135,224	6,587,790	6,236,897	6

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

GENERAL

Since its inception, the activities of the Company have consisted primarily of obtaining the rights for developing and marketing proposed pharmaceutical products, 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 December 31, 2001 of $54.1 million. In addition, the Company expects to incur additional losses from operations in 2002.

RECENT DEVELOPMENTS

On October 2, 2000 the Company announced the submission to the FDA of a new drug application (NDA) for Xyrem(R) (sodium oxybate) oral solution as a treatment for symptoms in narcolepsy. On March 2, 2001, the FDA granted a 90 day extension to the Company's NDA in order that the Company could submit additional data requested by the FDA. On June 6, 2001, the FDA held a meeting of the Peripheral and

Central Nervous System Advisory Committee to consider Xyrem. The Advisory Committee voted affirmatively that Xyrem was effective in treating cataplexy, but was split on the issue of Xyrem's safety due to the relatively small size of the safety database. As a result, the FDA issued an Approvable Letter for Xyrem on July 2, 2001. The Approvable Letter defined issues that required resolution before approval could be granted for the treatment of cataplexy. On October 23, 2001, the Company announced that the FDA had accepted the Company's response, in the form of an amendment, to the Approvable Letter. The amendment represented the Company's complete response to the issues contained in the FDA's Approvable Letter. The amendment includes revisions to product labeling and the risk management program, a safety update of ongoing clinical trials, and respiratory data collected during all night polysomnographic recordings of narcolepsy patients in a clinical trial completed in late 2000. The Company believes it has addressed all questions and issues the FDA may have regarding the NDA for Xyrem. The FDA assigned April 9, 2002 as its new action deadline for this NDA.

On December 10, 2001 the Company announced that it had raised $14.1 million in gross proceeds through a private placement of approximately 1.7 million shares of newly issued common stock at a price of $8.25 per share to Alta BioPharma Partners II, L.P., Alta Embarcadero BioPharma Partners II, LLC and funds managed by current investors OrbiMed Advisors LLC and Medical Strategy. The Company has registered the shares under the Securities Act of 1933, as amended. Thomas Weisel Partners LLC acted as the Company's agent and financial advisor in the transaction.

CRITICAL ACCOUNTING POLICIES

REVENUE RECOGNITION

Sales are recognized at the time a product is shipped to the Company's customers and are recorded net of reserves for estimated returns of expired product and discounts. The Company is obligated to accept from all domestic customers the return of products that have reached their expiration date. The Company is not obligated to accept exchange of outdated product from its international distribution partners. The Company monitors the return of product and modifies its accrual for outdated product returns as necessary. Management bases the reserve on historical experience and these estimates are subject to change.

ACCOUNTS RECEIVABLE ALLOWANCE

The Company determines an allowance amount based upon an analysis of the collectibility of specific accounts and the aging of the accounts receivable. There is a concentration of sales to larger medical wholesalers and distributors. The Company performs periodic credit evaluations of its customers' financial condition. Receivables are generally due within 30 days of the invoice date. Credit losses relating to customers have not been material since the Company's inception.

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.

RESULTS OF OPERATIONS

TWELVE MONTHS ENDED DECEMBER 31, 2001 VS. TWELVE MONTHS ENDED DECEMBER 31, 2000

Revenues increased from $11.2 million for the twelve months ended December 31, 2000 to $11.3 million for the twelve months ended December 31, 2001, an increase of $0.1 million or 1%. Sales of both Antizol and Busulfex exceeded revenue expectations in 2001. Busulfex continues to gain acceptance in major cancer treatment centers and Antizol has become a standard of care in the treatment of ethylene glycol and methanol poisonings. International sales of the Company's products decreased in 2001 due primarily to a delay in the shipment of clinical trial supply product requested by an international partner. The sales of Cystadane, Sucraid Antizol-Vet and Elliotts B met the Company's expectations in 2001 but are not expected to increase significantly in 2002. Revenues 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 Company's ability to optimize distribution of its approved products.

Cost of sales increased from $1.5 million for the twelve months ended December 31, 2000 to $1.6 million for the twelve months ended December 31, 2001, an increase of $0.1 million or 4%. This increase is primarily attributable to the increase in sales in 2001. The gross margins for both 2001 and 2000 were 86%. Cost of sales as a percentage of revenues 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 decreased from $6.8 million for the twelve months ended December 31, 2000 to $4.9 million for the twelve months ended December 31, 2001, a decrease of $1.9 million or 27%. The decrease is the result of reduced research and development spending on Xyrem during 2001. The Company's efforts in 2001 were primarily focused on supporting the Xyrem NDA submission. Prior year spending included amounts for clinical trials included in the NDA submission. The Phase III(b) trial for Xyrem now underway will increase research and development spending in subsequent quarters as will additional trials and data updates requested by the FDA. Clinical spending for these activities will be dependent on a number of factors, including among others, the number of human subjects screened and enrolled in the trials, and the number of active clinical sites.

Sales and marketing expense increased from $5.6 million for the twelve months ended December 31, 2000 to $6.3 million for the twelve months ended December 31, 2001, an increase of $0.7 million or 12%. This increase is largely attributable to significantly higher spending for pre-approval marketing activities relating to Xyrem. The Company expects sales and marketing expenses to increase further in 2002 because of additional marketing and sales efforts for the anticipated commercialization of Xyrem in 2002.

General and administrative expense increased from $4.1 million for the twelve months ended December 31, 2000 to $4.8 million for the twelve months ended December 31, 2001, an increase of $0.7 million or 17%. Approximately 34% of the increase in general and administrative expenses is related to compensation expense associated with stock options. The balance of the increase is related to building infrastructure, including the addition of staff to prepare for the anticipated launch of Xyrem. General and administrative expenses are expected to increase above current levels in subsequent quarters.

Other income is interest income from investment activities. Other income decreased from $0.8 million for the twelve months ended December 31, 2000 to $0.3 million for the twelve months ended December 31, 2001. This decrease is the

result of cash used to fund development and working capital activities of the Company. In addition, interest rates on invested funds have been declining, reducing the yields received. Other income is expected to increase in 2002 as a result of the additional cash made available by the equity transaction completed in December 2001.

Preferred stock dividends relate to shares of Senior Convertible Preferred Stock issued on July 23, 1998 and shares of Series B Convertible Preferred Stock issued on August 2, 1999. Both classes have dividend rates of 7.5%. Preferred stock dividends were $0.9 million for the twelve months ended December 31, 2001 and 2000. Preferred stock dividends, which commenced on February 1, 1999 for the Senior Convertible Preferred and on February 1, 2000 for the Series B Convertible Preferred Stock, are payable in arrears on August 1 and February 1 of each year. The Company previously satisfied its dividend payment obligation by issuing additional preferred stock, as permitted by the terms of the Senior Convertible Stock. The Company intends to continue to satisfy its future dividend payment obligations by the issuance of unregistered common shares of stock for the Senior Convertible Preferred Stock and additional shares of preferred stock for the Series B Convertible Preferred Stock, which will cause preferred stock dividends to increase in subsequent quarters.

Net loss applicable to common stockholders was $(6.9) million for the twelve months ended December 31, 2001 compared to a net loss of $(7.0) million for the twelve months ended December 31, 2000. Basic and diluted loss per common share for these respective periods were $(0.80) and $(0.86), based on weighted average number of common shares outstanding of 8,597,331 and 8,135,224, respectively.

TWELVE MONTHS ENDED DECEMBER 31, 2000 VS. TWELVE MONTHS ENDED DECEMBER 31, 1999

Revenues increased from $6.5 million for the twelve months ended December 31, 1999 to $11.2 million for the twelve months ended December 31, 2000, an increase of $4.7 million or 73%. Sales of both Antizol and Busulfex exceeded expectations in 2000. Busulfex continues to gain acceptance in the major cancer treatment centers and Antizol has become a standard of care in the treatment of ethylene glycol and methanol poisonings. The Company expects Antizol revenues to decline slightly in 2001 because most hospitals that were expected to stock Antizol have done so, and future orders will most likely be based on use. International sales of the Company's products increased in 2000. The sales of Cystadane, Sucraid Antizol-Vet and Elliotts B met the Company's expectations in 2000 and are not expected to increase significantly in 2001. Revenues 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 Company's ability to optimize distribution of its approved products.

Cost of sales increased from $0.8 million or 12% of revenues for the twelve months ended December 31, 1999 to $1.5 million or 14% of revenues for the twelve months ended December 31, 2000, an increase of $0.7 million or 88%. The increase is primarily attributable to the increase in sales in 2000. Cost of sales as a percentage of revenues 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 from $5.0 million for the twelve months ended December 31, 1999 to $6.8 million for the twelve months ended December 31, 2000, an increase of $1.8 million or 36%. Development activities for Xyrem, principally clinical and toxicology spending, increased significantly over 1999 levels due to the commencement in 2000 of additional Xyrem clinical trials supporting additional future Xyrem claims and the completion of the

submission of the NDA for Xyrem on October 2, 2000.

Sales and marketing expense increased from $3.4 million for the twelve months ended December 31, 1999 to $5.6 million for the twelve months ended December 31, 2000, an increase of $2.2 million or 65%. This increase is largely attributable to significantly higher spending related to the pre-approval market activities related to Xyrem and on-going marketing activities for Busulfex. The Company expects sales and marketing expenses to increase further in 2001 because of the anticipated commercialization of Xyrem in 2001 and additional support for the currently approved products.

General and administrative expense increased from $2.8 million for the twelve months ended December 31, 1999 to $4.1 million for the twelve months ended December 31, 2000, an increase of $1.3 million or 46%. This increase related to building infrastructure, including the addition of staff to prepare for the anticipated launch of Xyrem. General and administrative expenses are not expected to increase significantly above current levels in subsequent quarters.

Other income is interest income from investment activities. Other income increased from $0.3 million for the twelve months ended December 31, 1999 to $0.8 million for the twelve months ended December 31, 2000. As a result of higher levels of invested funds from the proceeds of the private placement in February 2000, interest income has increased during 2000. Other income is expected to decrease during 2001 as invested funds are used to fund operations during the year.

Preferred stock dividends relate to shares of Senior Convertible Preferred Stock issued on July 23, 1998 and shares of Series B Convertible Preferred Stock issued on August 2, 1999. Both classes have dividend rates of 7.5%. Preferred stock dividends were $0.9 million for the twelve months ended December 31, 2001 and 2000. Preferred stock dividends, which commenced on February 1, 1999 for the Senior Convertible Preferred and on February 1, 2000 for the Series B Convertible Preferred Stock, are payable in arrears on August 1 and February 1 of each year. The Company previously satisfied its dividend payment obligation by issuing additional preferred stock, as permitted by the terms of the Senior Convertible Stock. The Company intends to continue to satisfy its future dividend payment obligations by the issuance of unregistered common shares of stock for the Senior Convertible Preferred Stock and additional shares of preferred stock for the Series B Convertible Preferred Stock, which will cause preferred stock dividends to increase in subsequent quarters.

Net loss applicable to common shareholders was $(7.0) million for the twelve months ended December 31, 2000 compared to a net loss of $(5.9) million for the twelve months ended December 31, 1999. Basic and diluted loss per common share for these respective periods were $(0.86) and $(0.90), based on weighted average number of common shares outstanding of 8,135,224 and 6,587,790, respectively.

LIQUIDITY AND CAPITAL RESOURCES

Since its formation in July 1994, the Company has financed its operations principally from net proceeds from several public and private financings, interest income and product sales. The various public and private placement transactions since inception resulted in aggregate net proceeds, after commissions and expenses, of $60.5 million. These net proceeds include the private placement of 1.7 million shares of newly issued common stock with net proceeds of $13.0 million in December 2001.

Net working capital (current assets less current liabilities) increased to

$18.0 million at December 31, 2001 from $10.3 million at December 31, 2000. Cash, cash equivalents, and available-for-sale securities increased from $11.4 million at December 31, 2000 to $19.0 million at December 31, 2001. The Company invests excess cash in short-term, interest-bearing, investment grade securities.

The Company has a commercial revolving line of credit with a bank which expires on June 15, 2002. The maximum amount available to the Company under this line of credit is $1,000,000, subject to certain limitations based on the Company's cash collateral. The Company intends to renew this line of credit facility. However the Company cannot assure that the bank will do so, or that it will do so on terms acceptable to the Company. In connection with a financing transaction completed in August 1999, the Company received a $2.05 million commitment in the form of a line of credit from UBS Capital. This line was eliminated in connection with a financing transaction in December 2001. The Company had not borrowed under the UBS Capital agreement. In addition, the Company has not borrowed under the bank arrangement.

The Company's commitments for outside development spending increased to $4.0 million at December 31, 2001 from $3.9 million at December 31, 2000. The increase is principally attributable to the timing of the initiation of clinical trials for Xyrem development activities. The Company expects development spending to increase as the Xyrem Phase III(b) clinical trial progresses and post approval surveillance studies are completed. In addition, the Company continues to look at new product opportunities and any new initiatives will increase development spending.

The Company expects spending in 2002 for research and development, sales and marketing, and administration to increase significantly over 2001 levels. Management believes the Company's current working capital and anticipated operating cash flows from product sales will be sufficient to fund its operations through December 31, 2002.

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 tangible assets in excess of $4.0 million or (2) a market capitalization of at least $50.0 million. Net tangible assets are defined as total assets less the sum of total liabilities and intangible assets. The Company met both of the thresholds at December 31, 2001. The Company's net tangible assets at December 31, 2001 equaled approximately $18.4 million and the Company's market capitalization was approximately $135.0 million (based on the last sale price of $13.15 and 10,263,961 shares outstanding as of December 31, 2001). Although the Company does not expect to be profitable in 2002, the Company nevertheless expects to continue to meet the net tangible asset requirement for listing on the NASDAQ National Market. However there can be no assurance that the Company will continue to have adequate capital to meet the net tangible asset requirement through the year 2002 and thereafter. The NASDAQ National Market issued new listing qualifications, which will become effective November 2002, and which will replace the net asset requirement with a minimum net equity requirement of $10.0 million. At December 31, 2001, the Company meets the new listing requirements.

In connection with the 1998 and 1999 private placements of convertible preferred stock, the Company agreed to certain restrictions and covenants that 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.

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

The Company's investments consist of debt securities with contractual maturities of less than one year. Therefore, the Company does not believe its operations are exposed to significant market risk relating to interest rates.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The financial statements of the Company as of and for the year ended December 31, 2001 begin on page F-1 of this Annual Report.

ITEM 9. CHANGES AND DISAGREEMENTS WITH ACCOUNTANTS AND FINANCIAL DISCLOSURE

None.

PART III

ITEM 10. DIRECTORS AND EXECUTIVE OFFICERS OF THE REGISTRANT

(a) Directors of the Registrant.

The information required by this item is incorporated by reference from the information under the caption "Election of Directors" contained in the Company's Proxy Statement to be filed with the Securities and Exchange Commission in connection with the solicitation of proxies for the Company's Annual Meeting of Stockholders to be held on May 23, 2002 (the "Proxy Statement").

(b) Executive Officers of the Registrant.

The executive officers of the Company and their ages as of March 1, 2002 are as follows:

NAME	AGE	TITLE
John Howell Bullion.....	50	Chief Executive Officer and Chairman of the Board
William Houghton, M.D.....	59	Chief Operating Officer
Dayton T. Reardan, Ph.D.....	46	Vice President of Regulatory Affairs
Pamela J. Stahl.....	36	Vice President of Commercial Operations
Timothy G. McGrath.....	37	Vice President and Chief Financial Officer

Executive officers of the Company serve at the discretion of the Board of Directors with no fixed term. There are no family relationships between or among any of the executive officers or directors of the Company.

Mr. Bullion has been Chief Executive Officer of the Company since June 24, 1994 and Chairman of the Board of Directors since December 30, 1998. Mr. Bullion is a co-founder of Chronimed Inc., the company from which Orphan Medical, Inc. was spun-off in 1994. Prior to joining the Company, Mr. Bullion served as President of Bluestem Partners, an investment and consulting company, as President of Dahl & Associates, a soil and ground water remediation company and President of Concurrent Knowledge Systems, Inc., a software development company. Mr. Bullion also served as partner and Vice President with First Bank System Venture Capital Company for seven years.

Dr. Houghton has been the Company's Chief Operating Officer since August

1998. Prior to joining the Company, Dr. Houghton was employed in a variety of positions at Iotek, Inc. from April 1995 to August 1998, most recently as Chief Scientific Officer and Vice President of Clinical and Regulatory Affairs. At Iotek, Dr. Houghton was responsible for all research activities, regulatory and clinical research, and served as the medical liaison with Iotek's Medical Advisory Board. From February 1984 to March 1995, Dr. Houghton held a variety of management positions with Abbott Australasia and Abbott Laboratories in the United States.

Dr. Reardan has been the Company's Vice President of Regulatory Affairs since May 1995 and had been the Director of Regulatory Affairs since joining the Company in 1994. From 1993 to 1994, Dr. Reardon was Director of Development at CV Therapeutics. From 1984 to 1993, Dr. Reardon held a variety of management positions at Xoma Corporation.

Ms. Stahl has been the Company's Vice President of Commercial Operations since October 2001. From February 2000 to September 2001, Ms. Stahl held a number of positions at America TeleCare, Inc., most recently as Vice President of Sales where she had responsibility for sales, marketing, and distribution. From 1992 through January 2000, Ms. Stahl held several management positions in sales, managed care, and sales training at AstraZeneca L.P. where she was a member of the team that launched Prilosec(R), the leading treatment of acid related disorders. Ms. Stahl has also worked at Merck & Co., Inc. in sales and training positions supporting Zocor(R) and Pepcid(R).

Mr. McGrath has been the Company's Vice President and Chief Financial Officer since October 1999. Mr. McGrath had worked as consultant providing financial services to growing companies in the Minneapolis and Saint Paul area. From 1994 to 1998, he was Vice President of Finance at E. W. Blanch Holdings, Inc., a

publicly traded provider of integrated risk management and distribution services. Prior to joining E.W. Blanch Holdings, Mr. McGrath was with Ernst & Young LLP in Minneapolis, Minnesota.

(c) Compliance with Section 16(a) of the Securities Exchange Act of 1934.

The information required by this item is incorporated by reference from the information under the caption "Section 16(a) Beneficial Ownership Reporting Compliance" contained in the Proxy Statement.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this item is incorporated by reference from the information under the caption "Executive Compensation" contained in the Proxy Statement.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT

The information required by this item is incorporated by reference from the information under the caption "Security Ownership of Certain Beneficial Owners and Management" contained in the Proxy Statement.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS

The information required by this item is incorporated by reference from the information contained under the caption "Certain Relationships and Related Transactions" contained in the Proxy Statement.

PART IV

ITEM 14. FINANCIAL STATEMENT SCHEDULES AND REPORTS ON FORM 8-K

(a)(1). Financial Statements

(a)(2). Financial Statement Schedules

The following Financial Statement Schedule should be read in conjunction with the Audited Financial Statements referred to under Item 14(a)(1) above. Financial statement schedules other than those provided have been omitted since they are not required or are not applicable or the required information is shown in the financial statements or related notes.

(a)(3). Listing of Exhibits

EXHIBIT NUMBER	DESCRIPTION	METHOD OF FILING
3.1	Certificate of Incorporation of Orphan Medical, Inc. ("OMI")	*
3.2	Bylaws of OMI	*
4.1	OMI 1994 Stock Option Plan	(1)
4.2	OMI Employee Incentive Stock Option Agreement	(1)
4.3	OMI Non-Incentive Stock Option Agreement	(1)
4.4	OMI Non-Incentive Stock Option Agreement for Non-Employee Directors	(1)
4.5	Specimen Stock Certificate of OMI Common Stock	*
10.1	Marketing and Distribution Agreement between OMI and Chronimed effective July 2, 1994	(1)
10.2	Transfer Agreement between OMI and Chronimed effective July 1, 1994	(1)

10.3	Distribution and Spin-off Agreement between OMI and Chronimed effective July 2, 1994.....	(1)
10.4	Administrative Services Agreement between OMI and Chronimed effective July 2, 1994.....	(1)
10.5	Security Agreement between OMI and Chronimed effective July 2, 1994.....	(1)
10.6	Aminocaproic Acid License Agreement between Chronimed and Virginia's Center for Innovative Technology dated September 17, 1993.....	(1)
10.7	Patent and Technology License Agreement for Busulfan between Chronimed and The University of Texas M.D., Anderson Cancer Center, the Board of Regents of the University of Texas System and the University of Houston effective February 14, 1994.....	(1)
10.8	Letter Agreement regarding L-Cycloserine between Chronimed and Dr. Meier Lev dated December 29, 1993.....	(1)

EXHIBIT NUMBER	DESCRIPTION	METHOD OF FILING
10.09	Sublicense Agreement regarding 4-Methylpyrazole between Chronimed and Mericon Investment Group, Inc. dated December 17, 1993.....	(1)
10.10	License Agreement regarding Short Chain Fatty Acids between Chronimed and Richard Breuer dated March 2, 1994.....	(1)
10.11.1	Employment Agreement between OMI and John Howell Bullion dated October 29, 1999.....	(16)
10.12	Employment Agreement between OMI and Bertram A. Spilker, Ph.D., M.D. dated August 31, 1994.....	(1)
10.13	Assumption Agreement and Consent to Assignments regarding Short Chain Fatty Acids between OMI and Richard Breuer dated September 30, 1994.....	(2)
10.14	Assumption Agreement and Consent to Assignment regarding Aminocaproic Acid between OMI and Virginia's Center for Innovative Technology dated September 30, 1994.....	(2)
10.15	Assumption Agreement and Consent to Assignment regarding 4-Methylpyrazole between OMI and Mericon Investment Group, Inc. dated October 5, 1994.....	(2)
10.16	License Agreement regarding 4-Methylpyrazole between Kenneth McMartin and Mericon Investment Group, Inc. dated July 6, 1993.....	(2)
10.17	License Agreement regarding Glucaric Acid between OMI and Ohio State University Research Foundation dated December 28, 1994.....	(2)
10.18	Manufacturing Development and Supply Agreement regarding Aminocaproic Acid between OMI and Lifecore Biomedical, Inc. dated December 21, 1994.....	(2)
10.19	Marketing Agreement regarding Cystagon between OMI and Chronimed dated October 19, 1994.....	(2)
10.20	Assumption Agreement and Consent to Assignment regarding Busulfan between OMI and the University of Texas, M.D., Anderson Cancer Center, the Board of Regents of the University of Texas System and the University of Houston dated October 18, 1994.....	(2)
10.21	License Agreement regarding Catrix between OMI and	

	Lescarden, Inc. dated October 28, 1994.....	(2)
10.22	License Agreement regarding Sucrase between OMI and Hartford Hospital dated December 30, 1994.....	(2)
10.23	Option to Acquire License regarding Tretinoin between OMI and James Hannan dated February 6, 1995.....	(2)
10.24	Consulting Agreement between OMI and William B. Adams dated November 15, 1994.....	(3)
10.27	Agreement regarding Cystagon between Chronimed and Mylan Pharmaceutical dated October 17, 1994.....	(2)
10.29	Agreement between OMI and David A. Feste effective July 1, 1995.....	(4)
10.30	Development and License Agreement regarding Choline Chloride between OMI and Alan Buchman, Donald J. Jenden, Marvin E. Ament and Mark D. Dubin dated May 11, 1995.....	(4)
10.31	Addendum to License Agreement regarding Short Chain Fatty Acids between OMI and Richard Breuer dated May 12, 1995.....	(4)
10.32	Addendum to Administrative Services Agreement between OMI and Chronimed dated August 2, 1995.....	(4)
10.33	Amendment to Aminocaproic Acid License Agreement between OMI and Virginia's Center for Innovative Technology dated September 17, 1993.....	(5)
10.34	Amendment No. 1 to Marketing and Distribution Agreement between OMI and Chronimed dated July 2, 1994.....	(5)

EXHIBIT NUMBER	DESCRIPTION	METHOD OF FILING
10.35	Amendment to Marketing Agreement regarding Cystagon between OMI and Chronimed dated October 19, 1994.....	(5)
10.36	IRS tax qualification letter dated January 10, 1996 regarding the favorable determination of the tax status of the OMI 401(k) Savings Plan.....	(5)
10.38	Form of License Agreement regarding Colloidal Bismuth Subcitrate between OMI and Josman Laboratories, Inc. dated March 4, 1996.....	(5)
10.39	Agreement between OMI and Chronimed dated June 3, 1996 to amend Marketing and Distribution Agreement dated July 2, 1996.....	(6)
10.40	Cystadane Agreement between the OMI and Chronimed dated October 11, 1996.....	(7)
10.41	License Agreement regarding alpha galactosidase A between OMI and Research Corporation Technologies, Inc. dated March 15, 1996.....	(8)
10.42	License Agreement regarding 5-fluorouracil between OMI and the University of Miami and its Department of Ohpthalmology dated December 6, 1996.....	(8)
10.43	Collaborative Development Agreement regarding Clonidine between OMI and Medtronic, Inc. dated November 27, 1996.....	(8)
10.44	Distribution Agreement between OMI and W. A. Butler Company dated November 26, 1996.....	(8)
10.45	Distribution Services Agreement between OMI and Cardinal Health dated June 1, 1997.....	(9)
10.46	Termination Agreement between OMI and Chronimed dated as of June 27, 1997.....	(10)
10.47	Loan Agreement and Security Agreement between OMI and	

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* Filed herewith.

(1) Incorporated by reference to the corresponding exhibit numbers in OMI's Registration Statement on Form 10 filed on August 31, 1994, Commission File No. 0-24760.

(2) Incorporated by reference to the corresponding exhibit numbers in OMI's Registration Statement on Form S-1 filed on March 3, 1995, Commission File No. 0-24760.

(3) Incorporated by reference to the corresponding exhibit number in OMI's Quarterly Report on Form 10-Q for the quarter ended December 30, 1994, Commission File No. 0-24760.

(4) Incorporated by reference to the corresponding exhibit numbers in OMI's Annual Report on Form 10-K filed for the year ended June 30, 1995, Commission File No. 0-24760.

(5) Incorporated by reference to the corresponding exhibit numbers in OMI's Registration Statement on Form S-1 filed on March 11, 1996, Commission File No. 0-24760.

(6) Incorporated by reference to the corresponding exhibit number in OMI's Quarterly Report on Form 10-Q for the quarter ended June 30, 1996, Commission File No. 0-24760.

(7) Incorporated by reference to the corresponding exhibit number in OMI's Quarterly Report on Form 10-Q for the quarter ended September 30, 1996, Commission File No. 0-24760.

(8) Incorporated by reference to the corresponding exhibit numbers in OMI's Annual Report on Form 10-K filed for the year ended December 31, 1996, Commission File No. 0-24760.

(9) Incorporated by reference to the corresponding exhibit number in OMI's Quarterly Report on Form 10-Q for the quarter ended March 31, 1997, Commission File No. 0-24760.

(10) Incorporated by reference to the corresponding exhibit numbers in OMI's Annual Report on Form 10-K filed for the year ended December 31, 1997, Commission File No. 0-24760.

(11) Incorporated by reference to the corresponding exhibit numbers in OMI's Quarterly Report on Form 10-Q for the quarter ended June 30, 1998, Commission File No. 0-24760.

(12) Incorporated by reference to the similarly described exhibit included with OMI's Current Report on Form 8-K dated December 7, 1998, Commission File No. 0-24760.

(13) Incorporated by reference to the similarly described exhibit included with OMI's Current Report on Form 8-K dated February 9, 1999, Commission File No. 0-24760.

(14) Incorporated by reference to the similarly described exhibit included with OMI's Annual Report on Form 10-K for the year ended December 31, 1998, Commission File No. 0-24760.

(15) Incorporated by reference to the similarly described exhibit included with OMI's Current Report on Form 10-Q for the quarter ended June 30, 1999, Commission File No. 0-24760.

(16) Incorporated by reference to the similarly described exhibit included with OMI's Annual Report on Form 10-K for the year ended December 31, 1999, Commission File No. 0-24760.

(b). Reports on Form 8-K

None.

(c). Exhibits

See Item 14(a)(3) above.

(d). Financial Statement Schedules

See Item 14(a)(2) above.

SIGNATURES

Pursuant to the requirements of Section 13 or 15 (d) of the Securities Exchange Act of 1934, the Registrant has duly caused this Annual Report on Form 10-K to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of Minnetonka, State of Minnesota, on April 1, 2002.

ORPHAN MEDICAL, INC.

By: /s/ JOHN HOWELL BULLION
John Howell Bullion
Chief Executive Officer

By: /s/ TIMOTHY MCGRATH
Timothy McGrath
Chief Financial Officer

Pursuant to the requirements of the Securities Exchange Act of 1934, this Annual Report on Form 10-K has been signed by the following persons in the capacities and on the date indicated.

NAME	TITLE	DA
/s/ JOHN HOWELL BULLION John Howell Bullion	Chief Executive Officer, Secretary (principal executive officer) and Director	April 1
* Farah Champsi	Director	March 2
* Michael Greene	Director	March 2
* Thomas King	Director	March 2
* W. Leigh Thompson, Ph.D. M.D.	Director	April 1

*	Director	March 3
Julius Vida, Ph.D., M.B.A.		
*	Director	March 2
William M. Wardell, Ph.D., M.D.		

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* John Howell Bullion, pursuant to the Powers of Attorney executed by each of the officers and directors above whose name is marked by a "*", by signing his name hereto, does hereby sign and execute this Annual Report on Form 10-K on behalf of each of the officers and directors in the capacities in which the name of each appears above.

REPORT OF INDEPENDENT AUDITORS

Board of Directors and Shareholders
Orphan Medical, Inc.

We have audited the accompanying balance sheets of Orphan Medical, Inc. as of December 31, 2001 and 2000, and the related statements of operations, changes in shareholders' equity, and cash flows for each of the three years in the period ended December 31, 2001. Our audits also included the financial statement schedule listed in item 14(a). These financial statements and schedule are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements and schedule based on our audits.

We conducted our audits in accordance with auditing standards generally accepted in the United States. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Orphan Medical, Inc. at December 31, 2001 and 2000, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2001, in conformity with accounting principles generally accepted in the United States. Also in our opinion, the financial statement schedule referred to above, when considered in relation to the basic financial statements taken as a whole, presents fairly in all material aspects the information set forth therein.

Minneapolis, Minnesota
February 15, 2002

ORPHAN MEDICAL, INC.

BALANCE SHEETS

	DECEMBER 31,	
	2001	2000
ASSETS		
Current assets:		
Cash and cash equivalents	$ 19,011,245	$ 1,115,319
Available-for-sale securities	--	10,301,935
Accounts receivable, less allowance for doubtful accounts of $25,000 and $116,200 for 2001 and 2000, respectively	1,645,749	1,578,544
Inventories	1,242,120	1,602,949
Prepaid expenses and other	63,662	221,240
Total current assets	21,962,776	14,819,987
Property and equipment:		
Property and equipment	1,056,642	968,118
Accumulated depreciation	(673,142)	(504,187)
	383,500	463,931
Other assets	--	12,967
Total assets	$ 22,346,276	$ 15,296,885
LIABILITIES AND SHAREHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 1,152,426	$ 1,758,807
Accrued royalties	204,790	151,025
Accrued compensation	1,065,662	806,839
Deferred revenues	431,310	500,850
Accrued expenses	1,097,881	1,336,437
Total current liabilities	3,952,069	4,553,958
Commitments		
Shareholders' equity:		
Senior Convertible Preferred Stock, $.01 par value; 14,400 shares authorized; 8,706 shares issued and outstanding	87	87
Series B Convertible Preferred Stock, $.01 par value; 5,000 shares authorized; 3,417 and 3,174 shares issued and outstanding	34	32
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; 1,500,000 shares authorized; 0 shares issued and outstanding	--	--
Common stock, $.01 par value; 25,000,000 shares authorized; 10,263,961 and 8,442,759 issued and outstanding	102,639	84,427
Additional paid-in capital	72,364,612	57,849,390
Accumulated deficit	(54,073,165)	(47,178,667)
Unrealized gain (loss) on available-for-sale securities	--	(12,342)
Total shareholders' equity	18,394,207	10,742,927

Total liabilities and shareholders' equity.....	$ 22,346,276	$ 15,296,885

See accompanying notes.

ORPHAN MEDICAL, INC.

STATEMENTS OF OPERATIONS

	FOR THE YEAR ENDED DECEMBER 31, 2001	FOR THE YEAR ENDED DECEMBER 31, 2000	FOR THE YEA ENDED DECEMBER 31 1999
Revenues.....	$11,274,110	$11,185,634	$ 6,457,406
Cost of sales.....	1,591,826	1,532,446	803,562
Gross profit.....	9,682,284	9,653,188	5,653,844
Operating expenses:			
Research and development.....	4,933,278	6,832,130	4,975,706
Sales and marketing.....	6,259,045	5,606,506	3,430,539
General and administrative.....	4,807,847	4,094,905	2,756,827
Loss from operations.....	(6,317,886)	(6,880,353)	(5,509,228
Other income:			
Interest, net.....	321,315	793,238	287,989
Net loss.....	(5,996,571)	(6,087,115)	(5,221,239
Less: Preferred stock dividends.....	903,053	872,024	682,872
Net loss applicable to common shareholders.....	$(6,899,624)	$(6,959,139)	$(5,904,111
Basic and diluted loss per common share applicable to common shareholders.....	$ (0.80)	$ (0.86)	$ (0.90
Weighted average number of shares outstanding.....	8,597,331	8,135,224	6,587,790

See accompanying notes.

ORPHAN MEDICAL, INC.

STATEMENTS OF CASH FLOWS

	FOR THE YEAR ENDED DECEMBER 31, 2001	FOR THE YEAR ENDED DECEMBER 31, 2000	FOR THE YEAR ENDED DECEMBER 31, 1999

OPERATING ACTIVITIES			
Net loss	$(5,996,571)	$ (6,087,115)	$(5,221,239)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	168,955	141,783	115,777
Compensatory options	239,887	196,390	51,196
Changes in operating assets and liabilities:			
Accounts payable	(606,381)	1,194,705	(22,714)
Accrued expenses and deferred revenue	4,492	679,283	(768,469)
Inventories	360,829	(1,057,406)	(432,818)
Accounts receivable and other	103,340	(502,963)	(124,996)
Net cash used in operating activities	(5,725,449)	(5,435,323)	(6,403,263)
INVESTING ACTIVITIES			
Purchase of property and equipment	(88,524)	(252,781)	(158,980)
Purchase of short-term investments	--	(27,778,944)	(8,735,566)
Maturities of short term investments	10,314,277	21,296,658	9,447,288
Net cash (used in) provided by investing activities	10,225,753	(6,735,067)	552,742
FINANCING ACTIVITIES			
Proceeds from common stock offering	12,994,122	10,692,154	--
Proceeds from the issuance of shares under the Employee Stock Purchase Plan	134,288	398,596	--
Proceeds from stock option and warrants	267,414	1,992,313	876,546
Net proceeds from Preferred Stock offerings	--	--	2,876,869
Preferred stock dividend	(202)	(3,032)	(995)
Common stock redemption	--	--	(676,563)
Net cash provided by financing activities	13,395,622	13,080,031	3,075,857
Increase(decrease) in cash and cash equivalents	17,895,926	909,641	(2,774,664)
Cash and cash equivalents at the beginning of year	1,115,319	205,678	2,980,342
Cash and cash equivalents at the end of year	$19,011,245	$ 1,115,319	$ 205,678
SCHEDULE OF NONCASH INVESTING AND FINANCING ACTIVITIES			
Warrants issued for line of credit	$ --	$ --	$ 82,000
Issuance of preferred stock dividends	897,725	841,999	588,000

See accompanying notes.

ORPHAN MEDICAL, INC.

STATEMENT OF CHANGES IN SHAREHOLDERS' EQUITY

	PREFERRED STOCK		COMMON STOCK		ADDITIONAL PAID-IN	ACCUMULATE
	SHARES	AMOUNT	SHARES	AMOUNT	CAPITAL	DEFICIT
Balance at December 31, 1998	7,500	$ 75	6,560,096	$ 65,601	$39,946,113	$(34,433,6
Net proceeds from private offering of 2,950 shares						

of Series B Convertible Preferred Stock.....	2,950	30	--	--	2,876,838	
Options and warrants exercised.....	--	--	173,834	1,738	874,809	
Repurchase of Chronimed stock.....	--	--	(127,723)	(1,277)	(675,286)	
Compensatory options.....	--	--	--	--	51,196	
Warrants issued for line of credit.....	--	--	--	--	82,000	
Preferred stock dividends.....	588	6	--	--	587,994	(588,9
Comprehensive loss:						
Net loss.....	--	--	--	--	--	(5,221,2
Unrealized loss on available-for-sale securities.....	--	--	--	--	--	
Subtotal -- comprehensive loss.....						
Balance at December 31, 1999.....	11,038	111	6,606,207	66,062	43,743,664	(40,243,8
Net proceeds from private offering of 1,365,000 shares of Common Stock...	--	--	1,365,000	13,650	10,678,504	
Options and warrants exercised.....	--	--	384,746	3,847	1,991,113	(2,6
Proceeds from Employee Stock Purchase Plan.....	--	--	86,806	868	397,728	
Options issued for services.....	--	--	--	--	196,390	
Preferred stock dividends.....	842	8	--	--	841,991	(845,0
Comprehensive loss:						
Net loss.....	--	--	--	--	--	(6,087,1
Unrealized loss on available-for-sale securities.....	--	--	--	--	--	
Subtotal -- comprehensive loss.....						
Balance at December 31, 2000.....	11,880	119	8,442,759	84,427	57,849,390	(47,178,6
Net proceeds from private offering of 1,706,999 shares of Common Stock...	--	--	1,706,999	17,070	12,977,052	
Options and warrants exercised.....	--	--	41,700	417	266,997	
Proceeds from Employee Stock Purchase Plan.....	--	--	19,210	192	134,096	
Compensatory options.....	--	--	--	--	239,887	
Preferred stock dividends.....	243	2	53,293	533	897,190	(897,9
Comprehensive loss:						
Net loss.....	--	--	--	--	--	(5,996,5
Unrealized loss on available-for-sale securities.....	--	--	--	--	--	
Subtotal -- comprehensive loss.....						

Balance at December 31, 2001	12,123	$121	10,263,961	$102,639	$72,364,612	$(54,073,1

See accompanying notes.

ORPHAN MEDICAL, INC.

NOTES TO FINANCIAL STATEMENTS
DECEMBER 31, 2001

1. BUSINESS ACTIVITY

Orphan Medical, Inc. (the "Company") acquires, develops, and markets products of high medical value intended to address inadequately treated or uncommon diseases within selected therapeutic areas segments. 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 operated within a single segment, pharmaceutical product development, and had six approved products commercially available in the United States and several foreign countries.

As of December 31, 2001, the Company had not completed product development, obtained required regulatory approvals or verified the market acceptance and demand for Xyrem(R) (sodium oxybate) oral solution, one of its three principal products. Antizol(R) (fomepizole) Injection, Busulfex(R) (busulfan) Injection and Xyrem are the Company's principal products. In February 1999, the U.S. Food and Drug Administration (the "FDA") approved the Company's New Drug Application ("NDA") for Busulfex and the Company began commercial shipments of Busulfex to distributors and wholesalers during the same month. In addition, a Treatment Investigational New Drug ("IND") application for Xyrem was approved by the FDA in December 1998 and the Company began shipping Xyrem in February 1999, for use in its Treatment IND clinical trials which continue at December 31, 2001. The Treatment IND allows the Company to seek payments for Xyrem used by patients enrolled in the Treatment IND clinical trials, as well as gain additional clinical safety data that are expected to support the Company's NDA filing for Xyrem. The Company submitted its NDA for Xyrem on October 2, 2000. The FDA granted the application priority review status giving the FDA a goal of completing its review within 180 days. The FDA issued an Approvable Letter on July 7, 2001. The Company's complete response was accepted by the FDA on October 23, 2001. The FDA's action deadline is April 2002.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

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.

REVENUE RECOGNITION

Sales are recognized at the time a product is shipped to the Company's customers and are recorded net of reserves for estimated returns of expired product and discounts. The Company is obligated to accept from all domestic customers the return of products that have reached their expiration date. The

Company is not obligated to accept exchange of outdated product from its international distribution partners. The Company monitors the return of product and modifies its accrual for outdated product returns as necessary. Management bases the reserve on historical experience and these estimates are subject to change.

Deferred revenue represents prepayment from customers for products not yet shipped.

CASH EQUIVALENTS AND AVAILABLE-FOR-SALE SECURITIES

The Company considers all highly liquid investments with remaining maturities of 90 days or less when purchased to be cash equivalents. The Company considers all highly liquid investments with remaining maturities of more than 90 days when purchased to be available-for-sale securities. Cash equivalents are carried at cost plus accrued interest, which approximates market value. The Company records unrealized gain or loss, if any, on available-for-sale securities as a separate component of shareholders' equity.

CONCENTRATION OF CREDIT RISK

The Company invests its excess cash in U.S. government agency securities, investment grade commercial paper, and other money market instruments and has established guidelines relative to diversification and maturities in an effort to maintain safety and liquidity. These guidelines are periodically reviewed to take advantage of trends in yields and interest rates. The Company has not experienced any significant losses on its cash equivalents or available-for-sale securities.

ACCOUNTS RECEIVABLE ALLOWANCE

The Company determines an allowance amount based upon an analysis of the collectibility of specific accounts and the aging of the accounts receivable. There is a concentration of sales to larger medical wholesalers and distributors. The Company performs periodic credit evaluations of its customers' financial condition. Receivables are generally due within 30 days of the invoice date. Credit losses relating to customers have not been material since the Company's inception.

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.

	DECEMBER 31,	
	2001	2000
Raw materials and packaging	$ 981,583	$1,213,464
Finished goods	260,537	389,485

$1,242,120	$1,602,949

PROPERTY AND EQUIPMENT

Property and equipment are stated at cost. Maintenance and repairs are expensed as incurred. Depreciation is computed using the straight-line method over the assets' estimated useful lives of three to seven years.

RESEARCH AND DEVELOPMENT COSTS

All research and development costs are charged to operations as incurred. Research and development costs consist principally of preclinical and clinical testing costs, certain salary and related expenses, bulk drug and drug product costs incurred in support of clinical testing and for validation lots required by the FDA, toxicology studies and various technical consulting costs.

GRANT AWARDS

The FDA Office of Orphan Drug Products and the Small Business Administration provide, upon application and approval, non-refundable grant awards in support of certain research and development activities. Cash proceeds collected pursuant to the terms of such grant awards are accounted for on a reimbursement basis. The Company has no such grants as of December 31, 2001.

INCOME TAXES

The Company accounts for income taxes using the liability method. Deferred income taxes are provided for temporary differences between the financial reporting and tax bases of assets and liabilities.

STOCK BASED COMPENSATION

The Company accounts for its stock option plans under the intrinsic-value-based method prescribed by Accounting Principles Board Opinion No. 25, "Accounting for Stock Issued to Employees." The Company has adopted the disclosure only provision of Statement of Financial Accounting Standards No. 123, "Accounting for Stock Based Compensation."

LOSS PER SHARE

Basic and diluted loss per common share applicable to common shareholders are based upon the weighted average number of Common Stock shares outstanding during the respective period. Basic loss per share excludes any dilutive effects of options, convertible senior preferred stock and warrants. Basic and diluted loss per share are the same for the reported periods because the effect of stock options, warrants, and convertible securities is anti-dilutive.

RECLASSIFICATIONS

Certain prior year balances have been reclassified in order to conform with the current year presentation. These reclassifications have no impact on net loss or shareholders' equity as previously reported.

3. AVAILABLE-FOR-SALE SECURITIES

The amortized cost and estimated market value of available-for-sale securities, all of which have contractual maturities of one year or less, are as follows:

	AMORTIZED COST	GROSS UNREALIZED GAINS	GROSS UNREALIZED LOSSES	ESTIMATED MARKET VALUE
As of December 31, 2000				
Commercial paper	$ 4,362,738	$ --	$ 5,140	$ 4,357,598
U.S. Government securities	5,951,539	--	7,202	5,944,337
	$10,314,277	$ --	$12,342	$10,301,935

4. OPERATING LEASES

The Company has a non-cancelable operating lease for office space that expires on October 31, 2003. The Company also has an operating lease for certain office equipment expiring April 2003. Future minimum lease payments, including current real estate taxes and operating expenses under the facility lease and the equipment lease are $287,500 and $224,500 for the years ended December 31, 2002 and 2003 respectively. Total rent expense was approximately $302,900, $239,100, and $142,600 for the years ended December 31, 2001, 2000, and 1999, respectively.

5. BORROWINGS

The Company has a commercial revolving line of credit with a bank, which expires in June 2002. The maximum amount available to the Company under this arrangement is $1,000,000, subject to certain limitations. The Company's indebtedness to the bank may not exceed the lesser of (1) 75 percent of the Company's trade accounts receivable that have been outstanding for 90 days or less or (2) $1,000,000. Advances are charged a variable rate of interest equal to the prime rate plus one half of a percent. Through December 31, 2001, the Company has not borrowed under this arrangement.

6. INCOME TAXES

As of December 31, 2001, the Company had net operating loss (NOL) carryforwards of approximately $42,237,000, credit for increasing research activities (the "R&D credit") carryforwards of approximately $359,000 and orphan drug credit carryforwards of approximately $8,915,000, available to reduce its future tax liabilities. These carryforwards will begin expiring after 2010. For the years ended December 31, 2001 and 2000, a valuation allowance of $23,954,000 and $21,418,000, respectively, has been recognized to offset the deferred tax assets related to these carryforwards.

No current income taxes have been provided for the years ended December 31, 2001, 2000 and 1999 as the Company had a loss for both financial reporting and tax purposes.

Significant components of the Company's net deferred tax assets are as follows:

	DECEMBER 31, 2001	DECEMBER 31, 2000
Deferred tax assets:		
Net operating loss carryforwards	$ 14,361,000	$ 12,737,000
R&D and orphan drug credit carryforwards	9,274,000	8,405,000
Inventory reserves	168,000	114,000
All other reserves	154,000	163,000
Deferred tax liabilities:		
Depreciation	(3,000)	(1,000)
Valuation allowance for deferred tax assets	(23,954,000)	(21,418,000)
Net deferred tax assets	$ --	$ --

As a result of the 1995 public stock offering, the Company exceeded the limits allowable under Section 382 of the Internal Revenue Code related to changes in ownership percentage which governs future utilization of NOL, R&D credit, and orphan drug credit carryforwards (collectively, "tax benefit carryforwards"). The effect of this occurrence is to limit the annual utilization of a portion of the Company's tax benefit carryforwards attributable to the period prior to the change in ownership. Should another change in ownership occur, future utilization of the Company's tax benefit carryforwards may be subject to additional limitations under Section 382.

7. EMPLOYEE BENEFIT PLANS

The Company maintains a 401(k) Savings Plan, which is funded by elective salary deferrals by employees. The Plan covers substantially all employees meeting minimum eligibility requirements. The Plan does not require mandatory contributions by the Company, but discretionary contributions may be made at the election of the Company. The Company has not made any provision for discretionary contributions to the Plan.

On January 4, 2000, shareholders approved the Orphan Medical, Inc. Employee Stock Purchase Plan to be funded by employee contributions, generally through payroll deductions. All employees are eligible subject to certain requirements. The purchase price is 85% of the lower of the average of the high and the low trade on the first and last trading day of each purchase period, defined as each calendar quarter. The Company reserved 200,000 shares of its common stock for future issuance at the Plan's inception. From the Plan's inception through December 31, 2001, there have been 106,016 shares issued under the Plan.

8. STOCK OPTIONS

The Company has one stock option plan for employees and non-employees, the 1994 Stock Option Plan (the "Plan"). The Plan provides the Company may grant employee incentive stock options and non-qualified stock options at a price of not less than 100% of fair market value. Options are exercisable as prescribed

by the Plan and expire up to fifteen years from the grant date for non-qualified stock options and up to ten years from the grant date for employee incentive stock options. At December 31, 2001, the Plan has 2,675,000 shares of Common Stock reserved for issuance.

Options outstanding were granted as follows:

	PLAN	
	OPTIONS OUTSTANDING	WEIGHTED AVERAGE EXERCISE PRICE
Balance at December 31, 1998	1,428,647	$ 5.66
Options granted	234,305	6.39
Options canceled	(22,500)	5.51
Options exercised	(168,834)	5.04
Balance at December 31, 1999	1,471,618	5.85
Options granted	40,750	11.08
Options canceled	(15,300)	10.85
Options exercised	(179,010)	5.10
Balance at December 31, 2000	1,318,058	6.05
Options granted	259,883	11.49
Options canceled	(9,263)	5.82
Options exercised	(41,700)	6.52
Balance at December 31, 2001	1,526,978	$ 6.97

The following table summarizes information about the stock options outstanding at December 31, 2001:

	OPTIONS OUTSTANDING			OPTIONS EXERCISABLE	
RANGE OF EXERCISE PRICES	NUMBER OUTSTANDING	WEIGHTED AVERAGE REMAINING CONTRACTUAL LIFE	WEIGHTED AVERAGE EXERCISE PRICE	NUMBER EXERCISABLE	WEIGHTED AVERAGE EXERCISE PR
$4.19-$5.00	533,500	2.83 years	$5.00	533,500	$5.00
$5.38-$6.75	398,395	3.81 years	5.96	371,695	5.95
$6.83-$18.31	595,083	8.49 years	9.41	346,577	8.45
$4.19-$18.31	1,526,978		$6.97	1,251,772	$6.24

Fully vested and exercisable options were 1,251,772, 1,098,258, and 1,107,518, as of December 31, 2001, 2000, and 1999, respectively. The weighted average exercise prices for the fully vested and exercisable options as of December 31, 2001, 2000, and 1999 were $6.24, $5.85, and $5.57 respectively.

PRO FORMA INFORMATION

The Company applies the intrinsic-value method in accounting for stock issued to employees and directors. Accordingly, compensation expense is recognized only when options are granted with a discounted exercise price. Any such compensation expense is recognized ratably over the associated service period, which is generally the option vesting period.

Pro forma net loss and loss per share information, as required by Statement of Financial Accounting Standards No. 123, "Accounting for Stock Based Compensation" (SFAS 123), has been determined as if the Company had accounted for employee stock options under the fair value method. The fair value of these options was estimated at grant date using a Black-Scholes option pricing model with the following assumptions for 2001, 2000, and 1999, respectively

	2001	2000	1999
Expected dividend yield	0.00%	0.00%	0.00%
Expected stock price volatility	73%	72%	57%
Risk-free interest rate	5.75%	6.00%	5.88%
Expected life of options	10 years	10 years	10 years

The weighted average fair value of the options granted in 2001, 2000, and 1999 was $9.33, $9.13, and $3.55, respectively, as computed as described above.

For purposes of pro forma disclosures, the estimated fair value of the options is amortized to expense over a four year average vesting period. The Company's pro forma net loss for 2001, 2000, and 1999 was $(7,932,893), $(7,687,948), and $(6,014,176), and pro forma net loss per share was $(0.92), $(0.95), and $(0.91), respectively.

The Company granted 15,000 options to purchase unregistered common stock to a consultant in December 2000. These options were granted at $13.6875, the closing price of the Company's common stock on the date of grant. The options vest under certain conditions. The fair value of these options is being charged to expense over the vesting period of the options.

9. SHAREHOLDERS' EQUITY

On July 23, 1998 the Company issued $7.5 million of Senior Convertible Preferred Stock (the "Preferred Shares") in a private placement. The Company realized net cash proceeds of $7.1 million from the sale of the Preferred Shares after the payment of related offering expenses. The Preferred Shares were initially convertible, at the option of the holders, into shares of the Company's Common Stock at a price equal to $8.50 per share. The August 1999 financing, as discussed in the following paragraph, triggered antidilution provisions relating to the $8.1 million of the Senior Preferred Stock held as of August 1 (after giving effect to the semi-annual in-kind dividend distributions), which resulted in a decrease in the conversion price of those shares from $8.50 to $8.14 per share. The Preferred Shares have anti-dilution protection and bear a dividend of 7.5% per annum, payable semi annually, which during the first two years may by paid either in cash or by issuing additional Preferred Shares. In the third year and thereafter, the dividend may be paid

either in cash or by issuing Common Stock valued at the then current market price. At the Company's option upon their maturity in July 2008, the Preferred Shares must be (a) converted into Common Stock, subject to a $3.0 million conversion fee payable in cash or by issuing additional Common Shares, or (b) redeemed for cash at $1,000 per share plus accrued dividends. The holders of the Preferred Shares are entitled to and have exercised their right to designate an individual to serve on the Company's Board of Directors.

On August 2, 1999, the Company completed a $5.0 million financing transaction in a private placement. The funding consisted of a purchase of 2,950 shares of the Company's Series B Convertible Preferred Stock for an aggregate purchase price of $2.95 million and a commitment of $2.05 million of debt in the form of a line of credit. The Company had not borrowed on this line of credit and it was eliminated as a part of the December 2001 financing transaction. The Series B Convertible Preferred Stock ("Series B Preferred Shares") may be converted prior to August 2, 2009 into shares of the Company's Common Stock at a price of $6.50 per share. The Series B Preferred Shares have anti-dilution protection and bear a dividend of 7.5% per annum, payable semi annually, which during the first two years may by paid either in cash or by issuing

additional Series B Preferred Shares. In the third year and thereafter, the dividend may be paid either in cash or by issuing Common Stock valued at the then current market price. At the Company's option upon their maturity in August 2009, the Series B Preferred Shares must be (a) converted into Common Stock, subject to a $1.2 million conversion fee payable in cash or by issuing additional Common Shares, or (b) redeemed for cash at $1,000 per share plus accrued dividends.

In conjunction with the issuance of the preferred shares, the Company agreed to several restrictions and covenants, and granted certain voting and other rights to the holders of the preferred shares. One of these restrictions is that the Company cannot incur additional indebtedness, except for indebtedness secured solely by our trade receivables, until the Company has profitable operations, subject to certain limitations. Another important restriction is that, without the approval of a majority of the preferred stockholders, the Company 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.

On February 23, 2000, the Company completed the sale of 1,265,000 shares of its Common Stock at a price of $8.00 per share. The Company received net proceeds of $9.7 million from the transaction and registered the shares under the Securities Act of 1933, as amended.

On February 25, 2000, the Company completed the sale of 100,000 shares of its Common Stock at a price of $10.00 per share. The Company received proceeds of $1.0 million from the transaction and registered the shares under the Securities Act of 1933, as amended.

On December 6, 2001, the Company completed the sale of 1,706,999 shares of its Common Stock at a price of $8.25 per share. The Company received net proceeds of $13.0 million from the transaction and registered the shares under the Securities Act of 1933, as amended.

10. STOCK WARRANTS

The Company issued warrants to the underwriter related to the Company's 1995 public stock offering to purchase 222,500 shares of Common Stock at a price of $5.20 per share. These warrants were exercised in 2000. In 1999, the Company issued warrants to the underwriter related to the Company's 1995 public offering to purchase 10,000 shares of Common Stock at $8.50 per share. At December 31, 2001, the Company had warrants outstanding to purchase 5,000 shares of Common Stock, all of which are currently exercisable.

In connection with the August 1999 financing, the Company issued two seven-year warrants. One of the warrants entitles the holder to receive, upon payment of the $2.05 million exercise price, either 2,050 shares of Series C Convertible Preferred Stock (which is similar to the Series B Convertible Preferred Stock and which is convertible to shares of the Company's Series D Non-Voting Preferred Stock at a conversion price of $6.50 per share) or 315,385 shares of Series D Non-Voting Preferred Stock (which is equivalent to common stock except that it has no voting rights) or a combination of Series C Convertible Preferred Stock and Series D Non-Voting Preferred Stock, so long as the combined purchase price for the shares does not exceed $2.05 million. The second warrant, issued in relation to the line of credit, entitled the holder to purchase 282,353 shares of Series D Non-Voting Preferred Stock at an exercise price of $4.25 per share. The value of the warrants was $82,000 and was amortized over the term of the line of credit to interest expense. All of these warrants are outstanding at December 31, 2001. These warrants are not exercisable until July 23, 2002.

11. RESEARCH AND DEVELOPMENT COMMITMENTS

The Company has various commitments under agreements with outside consultants, contract drug developers and manufacturers, technical service companies, and drug distributors. In addition, the Company has commitments under license and research agreements. The Company does not have any joint venture agreements nor does it have any arrangements to perform research and development for other parties. The

Company recognizes the costs associated with these commitments as incurred based on the accrual method of accounting. Expenditures associated with these commitments totaled approximately $3,000,000, $4,900,000, and $3,600,000 for the years ended December 31, 2001, 2000, and 1999, respectively. The Company's commitment to incur additional expenditures in subsequent periods for development activities totaled approximately $4,045,000 $3,907,000, and $1,602,000 at December 31, 2001, 2000, and 1999, respectively. Commitments for research and development expenditures will likely fluctuate from year to year depending on, among other factors, the timing of new product development, if any, and clinical trial activity.

12. CONTRACT TERMINATION FEE

The Company and Chronimed Inc. ("Chronimed") entered into an Agreement dated June 27, 1997, in which Chronimed agreed to terminate certain agreements that had been in existence since the spin-off of the Company from Chronimed in 1994. Among the terminated agreements was the Marketing and Distribution Agreement dated July 2, 1994, as amended, under which Chronimed had the exclusive right to market and distribute certain of Orphan Medical's products, including Busulfex and Antizol. In consideration for terminating these agreements, the Company agreed to pay Chronimed compensation equal to $2,500,000, consisting of cash and shares of the Company's Common Stock. On

February 9, 1999, the Company completed the acquisition from Chronimed of the remaining 127,723 unregistered shares of the Company's Common Stock. The Company paid Chronimed $338,281 on February 9, 1999 and $338,282 on March 31, 1999 to satisfy all of its obligations under the June 1997 Termination Agreement.

13. GEOGRAPHIC INFORMATION

The Company operates in two geographic regions, domestic and international. The Company's international sales are primarily in Europe and Canada. The Company has no assets outside of the United States. The following is a summary of net sales by geographic region for the years ended December 31, 2001, 2000 and 1999, respectively.

	2001	2000	1999
Domestic	$ 9,566,099	$ 9,226,618	$5,790,118
International	1,708,011	1,959,016	667,288
Total	$11,274,110	$11,185,634	$6,457,406

14. QUARTERLY FINANCIAL INFORMATION

The following are unaudited quarterly results of operations for the years ended December 31, 2001 and 2000.

	QUARTER ENDED			
	MARCH 31, 2001	JUNE 30, 2001	SEPTEMBER 30, 2001	DECEMBER 31, 2001
Revenues	$ 2,341,510	$ 2,424,843	$ 2,940,438	$ 3,567,319
Gross profit	2,051,844	1,990,264	2,531,306	3,108,870
Net loss	(1,390,474)	(1,709,289)	(1,833,281)	(1,063,527)
Less: Preferred stock dividends	221,129	224,383	228,366	229,175
Net loss attributable to common shareholders	(1,611,603)	(1,933,672)	(2,061,647)	(1,292,702)
Basic and diluted loss per common share	(0.19)	(0.23)	(0.24)	(0.15)

	QUARTER ENDED			
	MARCH 31, 2000	JUNE 30, 2000	SEPTEMBER 30, 2000	DECEMBER 31, 2000

Revenues	$ 2,742,456	$ 3,025,127	$ 2,610,662	$ 2,807,389
Gross profit	2,283,103	2,591,435	2,169,975	2,608,675
Net loss	(897,845)	(1,006,516)	(1,724,650)	(2,458,104)
Less: Preferred stock dividends	211,426	214,156	221,861	224,581
Net loss attributable to common shareholders	(1,109,271)	(1,220,672)	(1,946,511)	(2,682,685)
Basic and diluted loss per common share	(0.15)	(0.15)	(0.23)	(0.32)

SCHEDULE II -- VALUATION AND QUALIFYING ACCOUNTS

ORPHAN MEDICAL, INC.

DESCRIPTION	BALANCE AT BEGINNING OF PERIOD	ADDITIONS: CHARGED TO COSTS AND EXPENSES	ADDITIONS: CHARGED TO OTHER ACCOUNTS -- DESCRIBE	DEDUCTIONS -- DESCRIBE(1)	B
YEAR ENDED DECEMBER 31, 2001					
Reserves and allowances deducted from asset accounts:					
Allowance for doubtful accounts	$116,200	$ 56,408		$147,608	
Allowance for excess inventory	334,602	$158,395			
YEAR ENDED DECEMBER 31, 2000					
Reserves and allowances deducted from asset accounts:					
Allowance for doubtful accounts	$113,000	$ 3,200		$ --	
Allowance for excess inventory	376,593			41,991	
YEAR ENDED DECEMBER 31, 1999					
Reserves and allowances deducted from asset accounts:					
Allowance for doubtful accounts	48,620	64,380	--	--	
Allowance for excess inventory	497,200	--	--	120,607	

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(1) Recovery of amounts previously reserved.