

NORD[®]

National Organization
for Rare Disorders, Inc.[®]

NORD'S MISSION

The National Organization for Rare Disorders, Inc., (NORD) is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and services.

THE BEGINNING

NORD was launched in 1983 as an outgrowth of a unified effort by rare-disease support groups to encourage the pharmaceutical industry to develop new and better treatments for rare "orphan" disorders. At the time, companies were not willing to devote their resources to rare disorders because of the limited commercial potential for these therapies. Consequently, voluntary health agencies and support groups banded together to urge passage of federal legislation that would provide appropriate incentives for pharmaceutical companies to develop drugs for rare disorders. In 1983, these efforts resulted in passage of the *Orphan Drug Act*, and NORD came into existence as a national, non-profit voluntary health agency, dedicated to the welfare of individuals and family members affected by rare diseases.

NORD TODAY

Since its beginnings in 1983, NORD has grown and expanded its services for patients, families, support groups, and medical professionals. It is now a federation of more than 130 not-for-profit health organizations serving people with rare disorders. Each year, hundreds of thousands of affected individuals and their families, as well as support groups, health care and human service professionals, and advocates for people with disabilities rely on NORD's assistance, information, leadership, and services.



Annual Report 2003

NORD[®]

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*...out of the darkness,
into the light...[®]*



NORD

Programs and Services

INFORMATION AND REFERRAL SERVICES

NORD's primary mission is to provide information to patients, members of their families, the medical community, other professionals such as educators and social workers, and the public at large, to increase understanding and awareness of rare disorders. Its primary mission also includes providing referrals to appropriate organizations and agencies. For these purposes, NORD offers the following:

WEB SITE

NORD's web site (www.rarediseases.org) receives approximately 100,000 visits each month. It provides information about rare diseases, patient organizations, and current news of interest to the rare-disease community.

NORD's Rare Disease Database (RDB)

This database provides information in understandable language on more than 1,135 rare disorders for patients and their families. The reports can be downloaded from NORD's web site or sent through the mail to people who call or write to NORD.

NORD's Organizational Database (ODB)

This database provides descriptions and contact information for approximately 2,000 patient organizations, support groups, clearinghouses, registries, and government agencies that serve individuals and families affected by rare disorders. It is available for unlimited searching on NORD's web site. In addition, referrals are made over the phone or through the mail to people who do not have access to the web.

Publications

NORD's publications include *The NORD Guide to Rare Disorders*, a 900-page medical text for physicians published in 2003 by Lippincott, Williams & Wilkins; the *NORD Resource Guide*, which is a printed version of the Organizational Database; the *Orphan Disease Update* and *NORD Online* newsletters; and *The Physician's Guide* series of free booklets for medical professionals.

MEDICAL RESEARCH

There continues to be a serious shortage of funding for clinical research on orphan diseases. NORD attempts to fill this void by providing seed grants to academic scientists for research on potential new treatments for rare disorders,

advocating for increased government research funds, and referring affected individuals to clinical trials and genetic investigations. NORD's funding and advocacy have enabled researchers to develop breakthrough treatments that have dramatically improved the length and quality of life for thousands of children and adults with rare disorders.

PATIENT SERVICES

NORD helps individuals and family members who need assistance in accessing benefits and services in their local communities. A genetic counselor and a nurse are on staff to provide individualized help to people coping with complicated medical services.

MEDICATION ASSISTANCE PROGRAMS

NORD's Medication Assistance Programs provide certain prescription pharmaceuticals to needy uninsured patients who cannot afford to purchase them. In general, these are expensive drugs for chronic conditions. NORD also administers Expanded Access Programs for certain experimental drugs that are on the FDA priority review track, as well as limited and/or emergency access programs for certain medications that are in limited supply.

FAMILY NETWORKING PROGRAM

NORD's Family Networking Program puts individuals and/or family members who are affected by the same rare disorders in touch with each other. In addition to promoting mutual support and the exchange of information, "networking" promotes the formation of new voluntary health agencies for specific rare diseases.

ADVOCACY

NORD continues to play a vital, prominent role in Washington, DC, addressing public policy issues on behalf of families with rare diseases, particularly when government programs and policies do not adequately address their needs. Moreover, NORD has played a crucial role worldwide in increasing recognition of rare disorders and seeking to minimize their impact on the lives of patients and families.

National Organization for Rare Disorders, Inc.
Statement of Financial Position
December 31, 2003, and December 31, 2002

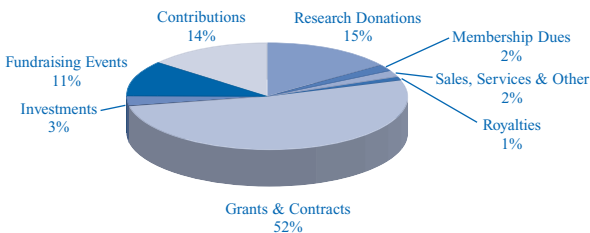
| | 2003 | 2002 |
|---|--------------------|------------------|
| ASSETS | | |
| <i>Current Assets:</i> | | |
| Cash and equivalents | \$5,117,306 | 3,997,902 |
| Investments | 1,028,698 | 872,517 |
| Accounts receivable, net | 210,892 | 135,555 |
| Prepaid expenses (and other current assets) | 131,856 | 8,202 |
| Total current assets | 6,488,752 | 5,014,176 |
| Property and equipment | 445,636 | 426,597 |
| <i>Less accumulated depreciation and amortization</i> | 356,904 | 328,250 |
| Net property and equipment | 88,732 | 98,347 |
| Security deposits | 18,062 | 18,062 |
| | 6,595,546 | 5,130,585 |
| LIABILITIES AND NET ASSETS | | |
| <i>Liabilities:</i> | | |
| Accounts payable and accrued expenses | 50,859 | 82,325 |
| Research grants payable | 776,833 | 534,384 |
| Total Liabilities | 827,692 | 616,709 |
| Net assets: | | |
| Unrestricted | 4,259,770 | 2,607,865 |
| Temporarily restricted | 1,508,084 | 1,906,011 |
| Total net assets | 5,767,854 | 4,513,876 |
| | \$6,595,546 | 5,130,585 |

Note: The Statements of "Financial Position" and "Activities" have been audited by a Certified Public Accountant.

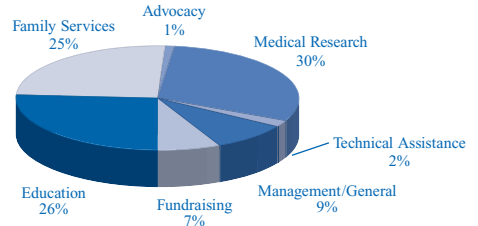
National Organization for Rare Disorders, Inc.
Statement of Activities for the Years Ended
December 31, 2003, and 2002

| | 2003 | 2002 |
|---|------------------|------------------|
| REVENUE AND SUPPORT | | |
| Indigent care drug programs | \$1,354,238 | 840,544 |
| Contributions and bequests | 834,380 | 1,365,609 |
| Special events revenue | 544,445 | 397,552 |
| Government and other grants | 394,910 | 529,352 |
| Investment incomes, including unrealized gains (losses) | 148,523 | 30,870 |
| Membership dues | 105,436 | 85,794 |
| Sales and distribution of materials and services to the public | 94,781 | 98,449 |
| Royalty income | 65,923 | 58,239 |
| Net assets released from restrictions | 1,738,202 | 1,343,524 |
| Total revenue and support | 5,280,838 | 4,749,933 |
| EXPENSES | | |
| <i>Program Services:</i> | | |
| Patient services | 906,410 | 632,127 |
| Education | 940,915 | 1,051,868 |
| Technical assistance | 81,089 | 17,809 |
| Research | 1,079,516 | 923,296 |
| Advocacy | 49,667 | 77,618 |
| Total program services | 3,057,597 | 2,702,718 |
| <i>Supporting services:</i> | | |
| Management and general | 319,842 | 340,181 |
| Fundraising | 161,070 | 191,980 |
| Direct cost of donor benefits | 90,424 | 48,290 |
| Total supporting services | 571,336 | 580,451 |
| Total expenses | 3,628,933 | 3,283,169 |
| Increase in unrestricted net assets | 1,651,905 | 1,466,764 |
| TEMPORARILY RESTRICTED NET ASSETS | | |
| Contributions and grants | 1,340,275 | 1,934,394 |
| Satisfaction of program restrictions | (1,738,202) | (1,343,524) |
| Increase (decrease) in temporarily restricted net assets | (397,927) | 590,870 |
| Change in net assets | 1,253,978 | 2,057,634 |
| Net assets-beginning of year | 4,513,876 | 2,456,242 |
| Net assets-end of year | 5,767,854 | 4,513,876 |

SOURCES OF FUNDS IN 2003



HOW FUNDS WERE DISBURSED IN 2003





20th ANNIVERSARY YEAR

Dear Friend of NORD:

We celebrated 20 years of progress during 2003, the 20th anniversary year for NORD and the *Orphan Drug Act*. At a gala in the spring, and throughout the year, we took time to reflect upon the achievements of the past two decades and look ahead to the challenges of the future.

NORD and the *Orphan Drug Act* are linked historically because they came into being at about the same time through the efforts of many of the same people. During the 10 years before the *Orphan Drug Act* was passed, only 10 new treatments were developed for rare diseases. In the 20 years since then, more than 1,100 new therapies have entered the research pipeline and 250 have been approved for marketing in the United States. This law has truly made a difference for people with rare diseases!

NORD has made a difference, too. Launched originally as a telephone and mail information clearinghouse, it has grown and added services over the years. Today, it maintains a news-oriented web site, administers medication assistance programs, provides publications for medical professionals, and oversees research grants and fellowships, all while continuing its core mission of education and referrals for patients and their families.

Highlights of 2003 include the following:

- NORD hosted a major conference on scientific, legal, regulatory, and economic issues related to generic biologics, and later published a white paper summarizing the proceedings.
- NORD administered 24 medication assistance programs for 18 pharmaceutical and biotechnology companies. These programs make expensive treatments available to uninsured patients who otherwise could not afford them. In 2003, NORD gave away more than \$38.2 million in drug assistance.
- NORD awarded 21 research grants and fellowships with a total value of \$768,134 during 2003, the highest number of any year to date. The grants provide seed money for promising research that might otherwise go unfunded.
- NORD added three new booklets to its free series for physicians. They are *The Physician's Guide to Pompe Disease*, *The Physician's Guide to Multiple System Atrophy*, and *The Physician's Guide to Hereditary Ataxia*.
- NORD's advocacy in Washington, DC, helped make possible the establishment, by the National Institutes of Health (NIH), of a Rare Diseases Clinical Research Network with \$51 million in grant funding over five years.
- NORD organized workshops in San Francisco and New York, sponsored by the NIH Office of Rare Diseases, to teach leaders of patient organizations how to access resources at the NIH.

Anniversaries help us focus on what has been accomplished...and what still remains to be done. The needs of rare-disease patients and families are many and complex. With your continued support, NORD will be able to meet those needs for many years to come.

Sincerely,

Abbey S. Meyers
President

James Broatch
Chairman, Board of Directors

2002 - 2003 Board of Directors



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