



Annual  
Report  
2012

Dear NORD Members and Friends:

We are proud to present this end-of-year report to you briefly summarizing the national leadership and representation that NORD provided during 2012 on behalf of the 30 million Americans with rare diseases.

In a year that saw the launch of a major new chapter in U.S. healthcare with the enactment of the *Affordable Care Act*, as well as significant scientific advances and renewed focus on innovative regulatory practices, NORD gave a voice to the entire rare disease community.

On the following pages, we briefly report specific initiatives and activities for the year in the following categories:

- Advocacy/Public Policy
- Awareness/Education
- Global Outreach
- Membership
- Research

## ENGAGE WITH NORD

We invite you to follow NORD's current activity in these and other areas on our website ([www.rarediseases.org](http://www.rarediseases.org)) where you can join us in advocacy and sign up for our monthly e-News and other updates. We also welcome your input, now or in the future. NORD exists to represent you, and your thoughts and suggestions are important to us.

Since 1983, NORD has served as the hub of a unique partnership involving the entire rare disease community. If you have not yet engaged in that partnership, we strongly encourage you to do so now.

With warm regards,



Peter L. Saltonstall, President and CEO



Michael D. Scott, Chair, Board of Directors

## ADVOCACY/PUBLIC POLICY

The seminal event of the year 2012 was the enactment of the *Affordable Care Act* in July 2012. NORD was actively engaged in the national debate about healthcare reform. Our advocacy focused on the following key elements of healthcare reform:

- Elimination of annual and lifetime health insurance caps
- Elimination of discrimination based on pre-existing medical conditions
- Elimination of the practice of discontinuing coverage for individuals who received dire diagnoses
- Establishment of an affordable health insurance option to assure coverage for all

While healthcare reform occupied center stage in the policy arena during 2012, NORD also provided leadership in other areas.

### **NORD was a Founding Member of the First-Ever Public-Private Partnership to Promote Development of Medical Devices**

At a press conference in December 2012, FDA Commissioner Margaret Hamburg, MD, and other thought leaders announced the establishment of a new Medical Device Innovation Consortium (MDIC). The purpose of MDIC is to promote faster development of innovative medical devices through collaboration of government, industry, the research community, and patients, as represented by NORD.

NORD President and CEO Peter L. Saltonstall spoke at the press conference and serves on the MDIC Board of Directors. NORD believes MDIC may provide a model for accelerated development of safe, effective therapies in other areas.

### **NORD was Honored for its Contributions to the Social Security Administration's Compassionate Allowances Program**

As Social Security Commissioner Michael Astrue approach the end of his tenure as Commissioner, he honored NORD for its support of SSA's Compassionate Allowances Program, which provides expedited review of applications for disability assistance for individuals with extremely disabling diseases, many of which are rare.

Since the program was established in 2008, NORD and its medical advisers have provided valuable support and assistance to SSA. Since Commissioner Astrue returned to private life in early 2013, NORD has remained in touch with SSA to assure continuance of this important program.

### **NORD Co-hosted with DIA the Second Annual U.S. Conference on Rare Diseases and Orphan Products**

This major conference drew together nearly 500 stakeholders from all segments of the rare disease community, including patient advocates, NIH and FDA senior officials, industry thought leaders, medical researchers, and investors.

## AWARENESS/EDUCATION

To promote awareness, connect the community, and facilitate early diagnosis and treatment, NORD provided numerous educational programs and services during 2012.

These included:

### **NORD Launched a New Online Platform for Physician Education and Awareness**

NORD physician guides at [www.nordphysicianguides.org](http://www.nordphysicianguides.org) provide information from medical experts to help physicians and other medical professionals become more aware of specific rare diseases. Launched in the spring of 2012, the new platform had received approximately 25,000 visits from physicians in nearly 100 countries by the end of the year.

### **NORD Created a New Blog for the Entire Rare Disease Community**

Topics of posts during 2012 included risk/benefit and related regulatory issues, the first-ever approved gene therapy, and expansion of the NIH Undiagnosed Diseases Program. The NORD blog may be found at - [blog.rarediseases.org](http://blog.rarediseases.org).

### **As National Sponsor for Rare Disease Day, NORD Achieved Many Successes**

Since 2009, NORD has served as the national sponsor in the U.S. for Rare Disease Day, working with global partners around the world. 2012 was the most successful observance of this awareness day to date, with the following highlights:

- A Sarah Lawrence graduate student worked with a mentor at NORD to develop a Rare Disease Day curriculum supplement for high school health and biology classes. The project drew good reviews from teachers, and became the subject of an invited poster presentation at the annual meeting of the National Society of Genetic Counselors (NSGC).
- FDA hosted its first-ever rare disease patient advocacy day in conjunction with Rare Disease Day. NORD assisted FDA in planning its event and arranged for a bus to take patient representatives to the FDA campus.
- Senators Sherrod Brown (OH) and John Barrasso (WY) submitted Senate Resolution 383 designating Feb. 29, 2012, “Rare Disease Day” across the U.S. Several members of Congress issued statements of support for the day.

## GLOBAL OUTREACH

### **NORD, EURORDIS and CORD Issued a Joint Declaration on Essential Components of Rare Disease Patient Registries**

At a time of broad global interest in rare disease patient registries, NORD collaborated with its partners in Europe, the European Organisation for Rare Diseases (EURORDIS) and in Canada, the Canadian Organization for Rare Disorders (CORD) to issue guidelines to protect and promote the best interests of patients.

## MEMBERSHIP

In line with its mission to mentor and encourage best practices among its approximately 200 member organizations, NORD hosted several regional meetings for patient organizations during 2012 with a grant from the Medtronic Foundation. These meetings, which provide an opportunity for members to learn from each other and share information, took place in Philadelphia, Chicago, Orlando, and San Francisco.

## RESEARCH

NORD's Research Program provides a conduit through which patients and patient organizations may fund research on diseases for which few other sources of funding may exist. NORD is grateful to its Scientific and Medical Advisory Committee for making this program possible.

During 2012, NORD awarded 12 grants in two cycles. Titles of all studies funded, and names and institutions of investigators, are available on the NORD website.

An article resulting from an earlier NORD grant was published in 2012 in the European Journal of Haematology.

## FINANCIAL DETAILS - 12/31/2012

### Assets

|                                                     |                     |
|-----------------------------------------------------|---------------------|
| Cash and equivalents . . . . .                      | 7,492,849           |
| Investments . . . . .                               | 303,736             |
| Accounts receivable, net . . . . .                  | 344,419             |
| Prepaid expenses and other current assets . . . . . | 54,319              |
| Net building, property and equipment . . . . .      | 2,114,138           |
| Other assets: . . . . .                             | 5,179               |
| <b>Total assets</b>                                 | <b>\$10,314,640</b> |

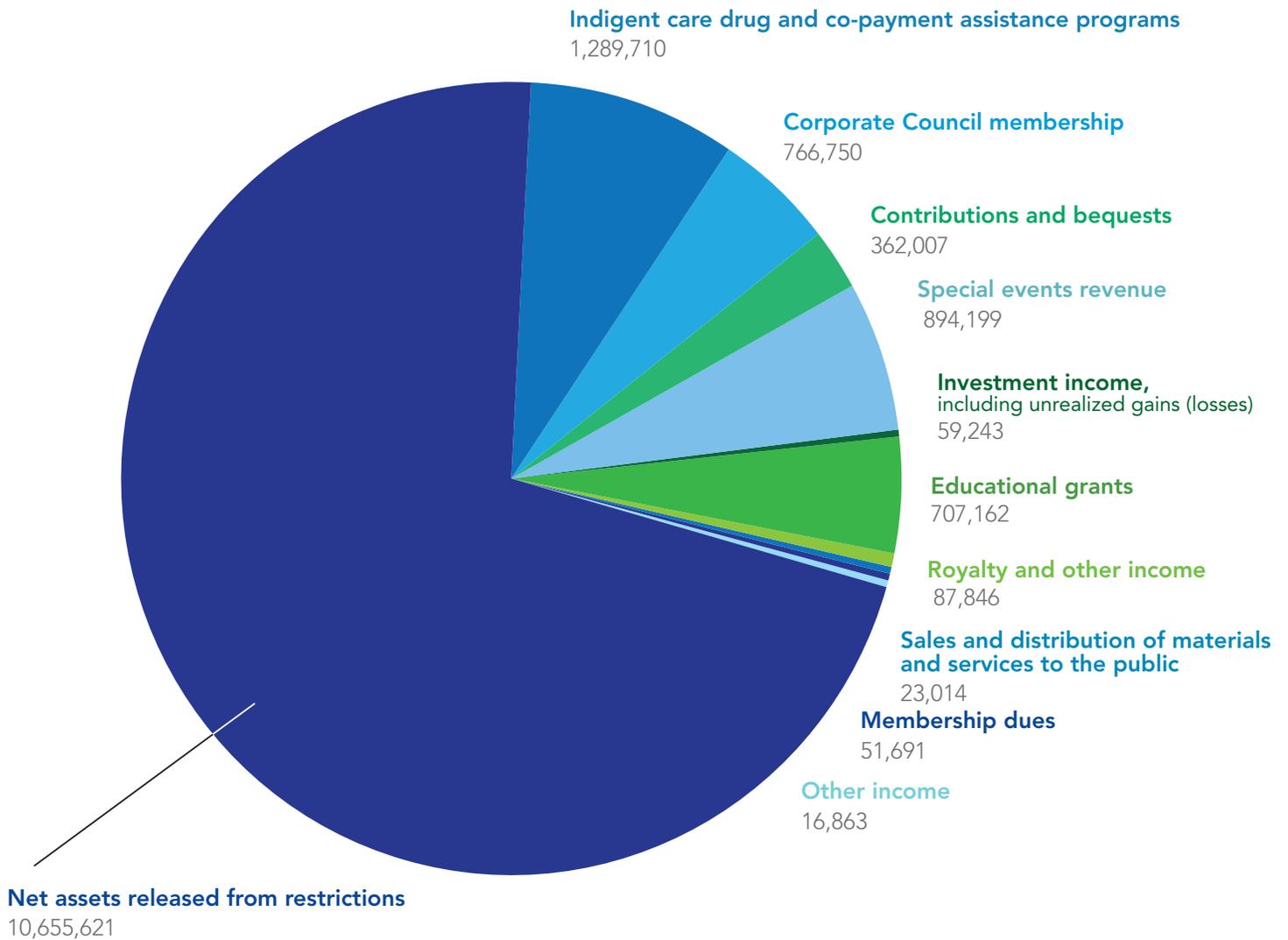
### Liabilities

|                                                       |           |
|-------------------------------------------------------|-----------|
| Current portion of long-term debt. . . . .            | 45,589    |
| Accounts payable . . . . .                            | 403,639   |
| Accrued expenses . . . . .                            | 1,208,553 |
| Research grants payable . . . . .                     | 841,947   |
| Deferred revenue . . . . .                            | 68,333    |
| Mortgage note payable, less current portion . . . . . | 647,455   |

### Net assets

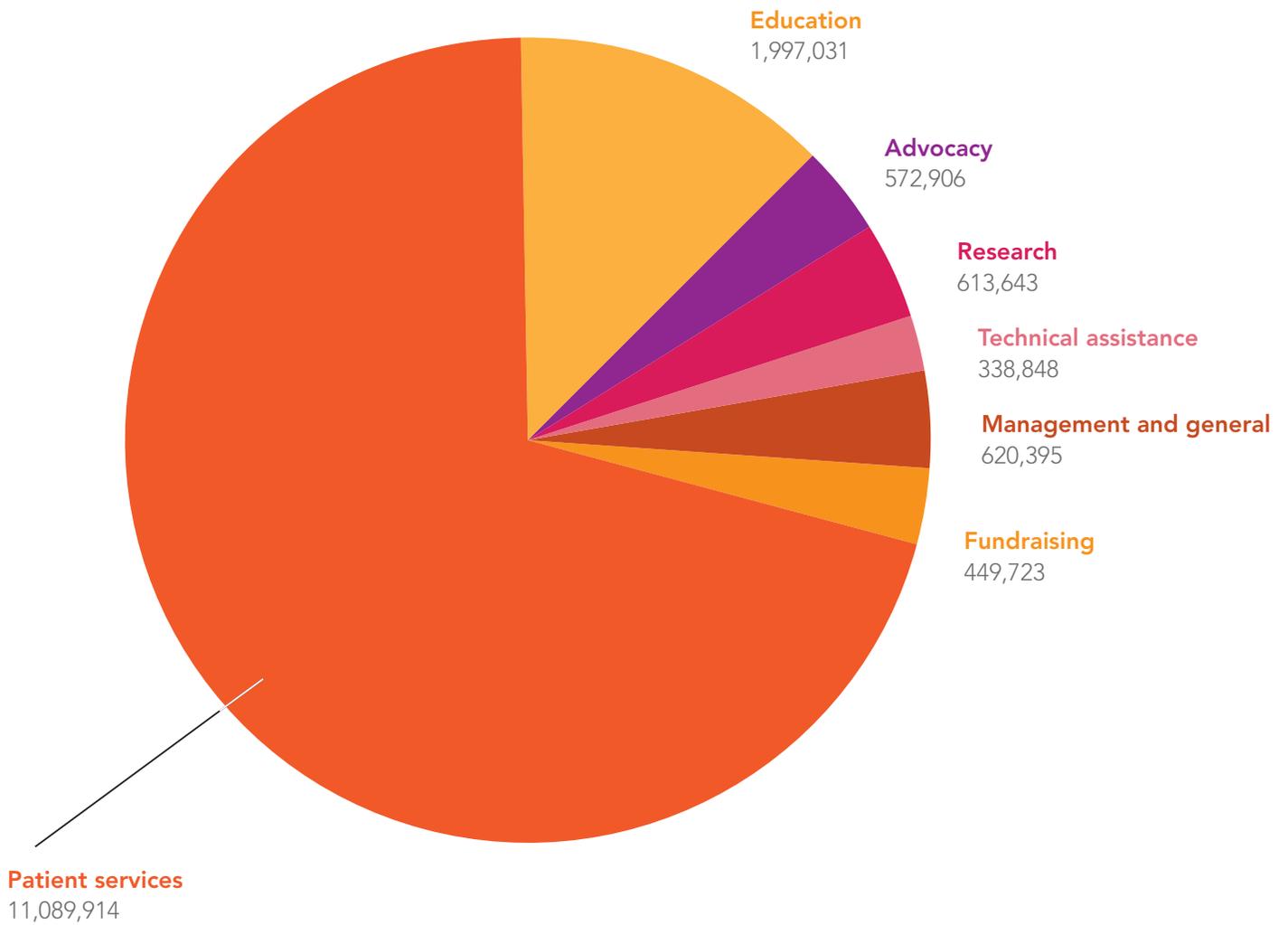
|                                           |                     |
|-------------------------------------------|---------------------|
| Unrestricted . . . . .                    | 4,484,557           |
| Temporarily restricted . . . . .          | 2,614,567           |
| <b>Total liabilities &amp; net assets</b> | <b>\$10,314,640</b> |

## REVENUE AND SUPPORT



Total revenue and support: \$14,914,106

EXPENSES



**Total expenses: \$15,682,460**  
Decrease in net assets (768,354)

## LOOKING AHEAD TO 2013: NORD'S 30TH ANNIVERSARY YEAR

As 2012 drew to a close, NORD senior leadership and staff were preparing for a year-long observance of the 30th anniversary of NORD and the Orphan Drug Act during 2013. NORD was founded by patient advocates who had worked together on behalf of the Orphan Drug Act. The year 2013 will be a time to highlight progress to date and renew our focus on the future. As always, NORD's mission remains to improve the lives of the 30 million Americans affected by rare diseases.