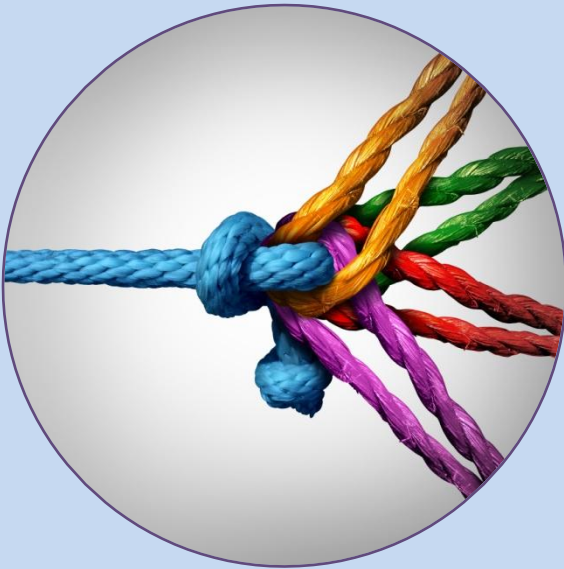


# *Annual Report 2017*



*Providing Education*

*Highlighting Resources*

*Creating Support Opportunities*

## *Rare New England*

**Coordinating Resources for Patients and Families  
With Rare and Complex Disorders**



## Executive Summary

Rare New England is pleased to present our 2017 Annual Report. Rare New England was previously known as Mito New England, which was founded in 2013 to allow patients and families find connections and emotional support among those with similar complicated medical problems. Rare New England herein referred to as (RNE) was established in 2016 by a group of medical professionals after recognizing the unmet needs of those affected by rare/complex/chronic disorders. The nonprofit strongly believes in empowering patients with information relevant to their medical issues through educational opportunities, enabling potential for improved quality of lives. RNE advocates for patients and families and seeks to offer educational opportunities to patients, families and healthcare professionals regarding how to achieve and maintain quality of life for patients and families, dealing with rare/ complex disease. RNE's goal is to empower patients and families around their own individual care needs.

RNE provides educational opportunities with group presentations by physicians, attorneys, bio-ethicists and other professions familiar with the Rare Community. RNE also provides for patients and families to learn about resources available to them by hosting speakers who represent wish granting organizations. RNE collaborates with many stakeholders in the many areas of rare disorders including, physicians across the United States and Canada, hospitals, state agencies, schools, advocacy groups, pharmaceuticals, patients, families and caretakers about the many aspects of rare disease treatment and support that must be improved. RNE also provides phone, e-mail, and in person support, both one-on-one and in a group setting.

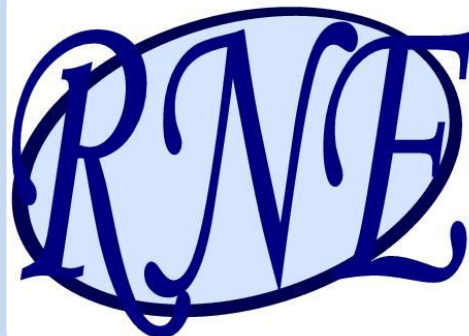
Rare New England has had no paid staff; therefore, the nonprofit relies on the effort and support of its board of directors, volunteers, and donors.

This report contains Rare New England's accomplishments throughout the year, future goals, financial information, testimonials, and photos.

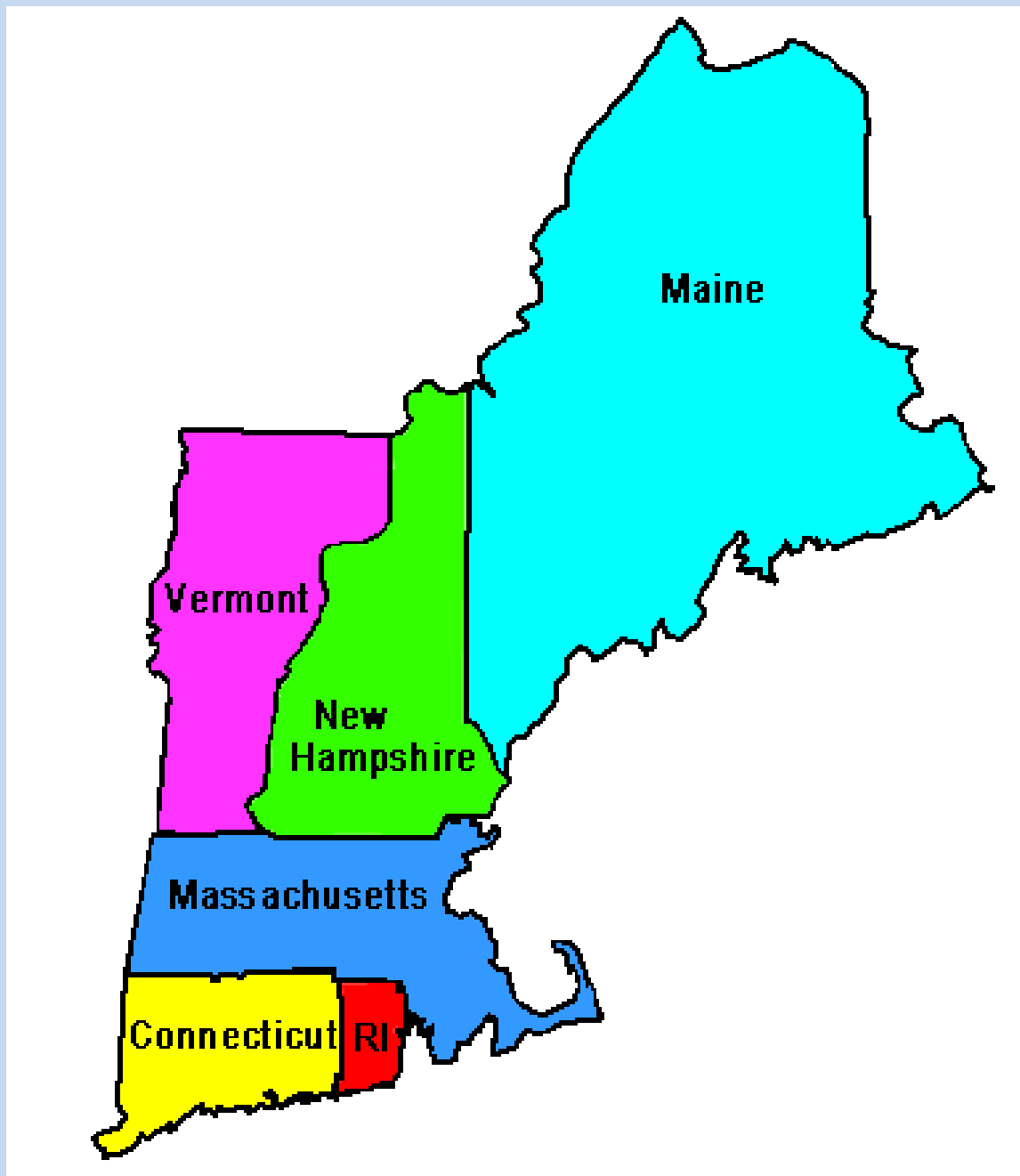
## Our Mission

Rare New England's Mission is to bring together New England patients, families and providers touched by rare and complex disorders. We offer educational opportunities, create awareness of available resources, and build foundations for support to improve patient quality of life.

RNE aims to bridge the gap between patients and families, clinics and clinicians, and the many resources available to them by existing government programs, advocacy organizations and patient groups, and state agencies, in the New England area.



**Rare New England, INC is based out of Attleboro, Massachusetts and strives to meet the needs of the whole New England area living and working with rare diseases including Maine, New Hampshire, Vermont, Massachusetts, Rhode Island and Connecticut.**



	<b>Office and Mailing Address</b> Rare New England, INC 120 North Main Street Suite 202 Attleboro, MA 02703

**Email Contact**  
Info@rarenewengland.org

**Phone Contact**  
508-699-4035

**Visit our website to learn more about whom we are and what we do.**  
**[www.rarenewengland.org](http://www.rarenewengland.org)**

**Find us on social media!**  
Facebook – [www.facebook.com/www.rarenewengland.org](https://www.facebook.com/www.rarenewengland.org)  
Twitter - #RareNewEngland  
Instagram - rarenewengland

## *RNE Board of Directors*



*Julie Gortze, RN  
President*



*Mark Korson, MD  
Director*



*Lois Foster, LICSW  
Secretary*



*Amel Karaa, MD  
Director*



*Janet Silva, MSF  
Treasurer*



*John C. Campbell, BS  
Director*

## **Acknowledgements**

RNE is grateful for the insight, support and efforts by the following RNE Committee Members and Volunteers; our accomplishments shine through their selfless efforts for the rare disease cause.

### **RNE Committee Members**

Debra McEleney

Rita Mauss

Allison Wood

### **Medical Advisory Board**

Richard Barnum, MD

Dmitriy Niyazov, MD

Pradeep Chopra, MD

Deborah Dreyfus MD MSc

Jessica Shriver, MA Theology, MS Bioethics

### **RNE Team Volunteers**

Heather Ruggiero

Lisa Deck

Allison Wood

Rachael Friend

Joanna Mechlinski

Erin Ruff

Melissa Hoyt



## ACCOMPLISHMENTS

2017 was a successful year for Rare New England. We continued to carry out our mission to bring together New England patients, families, and providers touched by rare and complex disorders: offering educational opportunities; creating awareness of available resources, and building foundations for support to improve patient quality of life. Rare New England was able to help hundreds of patients and families this year.



*Rare New England has accomplished much in just 1.5 years as a patient advocacy organization for those living and working with rare diseases.*

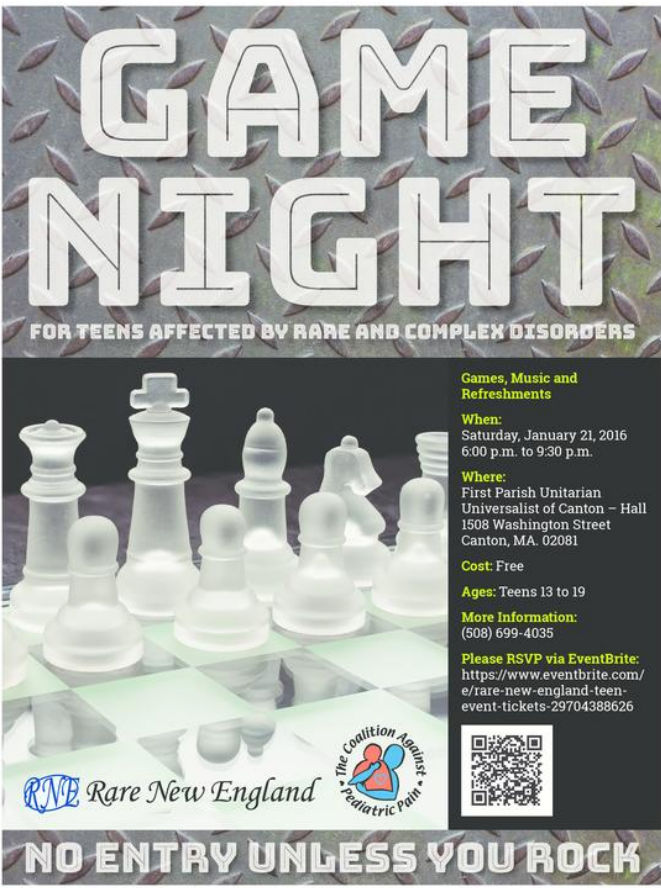
*Accomplishments that provide education, information & resources for the rare disease community include:*

- Hosting a 2-day Rare Disease Conference in Massachusetts.
- Hosting a Patient Conference in Vermont in partnership with University of Vermont Medical Center.
- Hosting a Teen Event in collaboration with Coalition Against Pediatric Pain.
- Hosting an event for a physical therapist to speak about exercise and the patient with a connective tissue disorder.
- Developing a Rare Disease Patient Track in partnership with the Festival of Genomics in Boston.
- Hosting a workshop at the Oley Foundation Annual Conference.
- Supporting rare disease legislative efforts in multiple states.
- Presenting at various events and conferences including Brandeis Genetic Counseling Program, New England Regional Genetics Group, US Pain Foundation, and more.
- Offering support to many patients and families through emails, phone calls and in-person meetings.
- Building and maintaining a website full of information.



With the help of donors and volunteers we were able to accomplish several significant milestones.

We organized multiple events that included hosting a teen game night for teens diagnosed with a rare disease, partnering with The Coalition Against Pediatric Pain;



**GAME NIGHT**  
FOR TEENS AFFECTED BY RARE AND COMPLEX DISORDERS

**Games, Music and Refreshments**

**When:** Saturday, January 21, 2016  
6:00 p.m. to 9:30 p.m.


**Where:** First Parish Unitarian Universalist of Canton - Hall  
1508 Washington Street  
Canton, MA. 02081



**Cost:** Free

**Ages:** Teens 13 to 19

**More Information:**  
(508) 699-4035

**Please RSVP via EventBrite:**  
<https://www.eventbrite.com/e/rare-new-england-teen-event-tickets-29704388626>



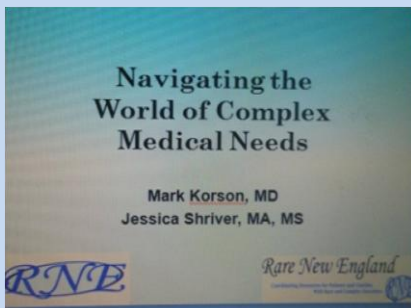
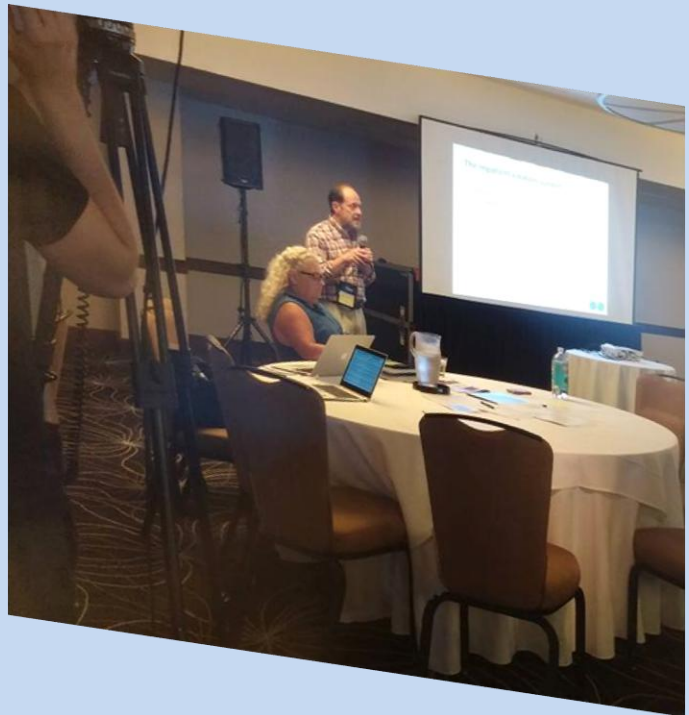
 

**NO ENTRY UNLESS YOU ROCK**

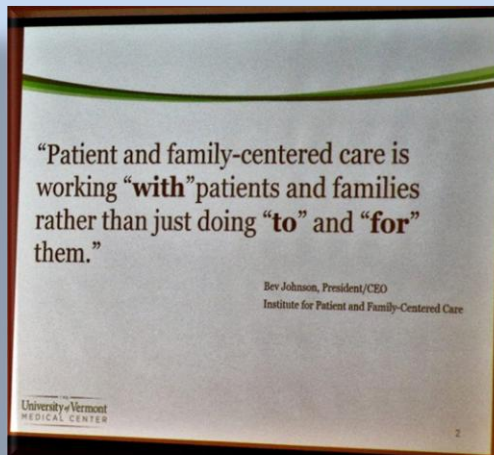




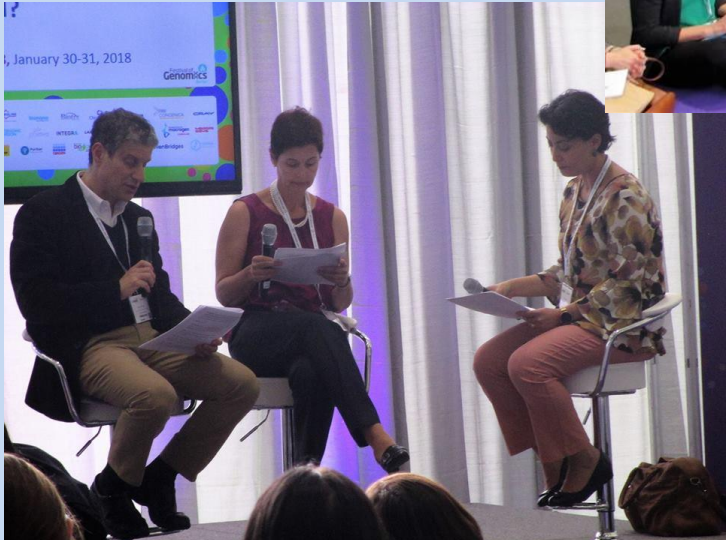
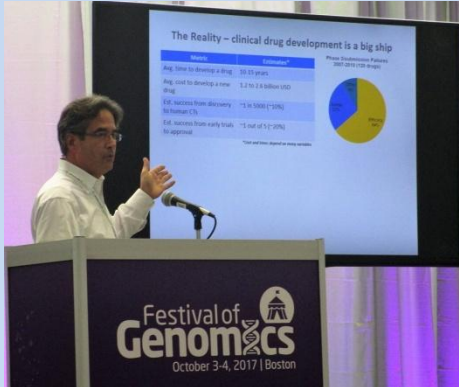
:hosting a workshop at the Oley Foundation Annual Conference called  
*“Communicating with your Medical Team”*;



:and hosting a conference in Partnership with the University of Vermont Children's Hospital called *"Improving Health Care Experiences in the Rare Disease Community with Information and Support"*.



Rare New England partnered with the Festival of Genomics in Boston to offer a Patient Track targeted toward patients and families living with rare diseases.



Rare New England has been collaborating with Massachusetts State Representative, Paul Heroux since 2015. This collaboration is focused on establishing a “Rare Disease Advisory Council” in Massachusetts. The rare disease advisory council would work to coordinate statewide efforts for the study of the incidences of rare diseases within the Commonwealth. The bill is known as HB 3714.

Legislative efforts are imperative in creating laws and setting framework for enforcement. Proper healthcare for all individuals requires strong legislation that paves the way for improved policies. There is a lot we can do to aid legislative efforts and we can begin with acts as simple as making telephone calls and/or sending emails to our own State Representatives and Senators to explain what it is like to live or work with rare disease and ask for their support for bills in play. Every call/email counts!

In words of a legislative aide who worked with Congressman Joseph Kennedy, "When we receive a call about an issue we take note. When we begin to receive multiple calls on an issue, we realize we have a problem to solve". Your call or email could be the one that makes the difference! Don't hold back; make that call today.



### Rare New England says thanks

Members from Rare New England held a volunteer appreciation luncheon Aug. 11 to thank volunteers for their commitment and services to patients and families living with rare diseases. Rep. Paul Heroux attended and provided an update on House Bill 3714, “An Act to Create a Rare Disease Advisory Council.”

He encouraged all attendees to urge their legislators to support the bill.



## GOALS




As Rare New England continues to grow we plan to continue to carry out our mission and help even more people. We have established goals for the upcoming 2018 year. These goals include the development of a Rare Disease Day Speaker Series, hosting an informational conference in a New England State, and develop a rare disease resource manual.

With the continued support of our incredible volunteers, hard-working board members, and new aid from additional donors, Rare New England hopes to provide more help for families afflicted with rare diseases as well as continuing to raise awareness for research of these diseases.

Rare New England desires to continue to grow and improve how it provides resources for those within the area. If individuals and their families continue to struggle against rare diseases, we will continue to help them get through their struggles. If this problem persists, we hope that Rare New England will not only persist along with it, but continue to grow.



**Rare New England**  
Coordinating Resources for Patients and Families  
With Rare and Complex Disorders 




Rare New England is proud to announce the launch of an annual speaker series –  
**"RNE Honors Rare Disease Day."**

During the week of Rare Disease Day (February 28th), RNE will host several forums in which patients and/or family members talk about their experiences to medical audiences at major teaching hospitals around New England. Their topics may include any combination of the following: their diagnostic journey, living with the disease, coping strategies, challenges in navigating the healthcare and/or educational systems, and more.

At each event, the patient presentation is preceded by a brief clinical overview of the disease given by a hosting physician; this provides the audience with a medical foundation to better understand the patient perspective.

These kinds of sessions provide health professionals and trainees the opportunity to see patients with disorders they might not otherwise encounter, making it more likely that they will recognize such patients in the future.

RNE is grateful for the time and effort generously provided by all the patient speakers. RNE Board Members, Mark Korson, MD, and Amel Karaa, MD, will serve as hosts for the Speaker Series.




**Our first year program will include talks at the following participating centers:**

- Yale New Haven Medical Center, New Haven, CT
- University of Vermont Medical Center, Burlington, VT
- Dartmouth-Hitchcock Medical Center, Lebanon, NH\*\*
- Boston University School of Medicine Genetic Counseling Program, Boston, MA\*\*
- Brandeis University Genetic Counseling Program, Waltham, MA\*\*
- Newton-Wellesley Hospital, Newton, MA


\*\*These events are not CME events and are generously sponsored by: TBA

[www.rarenewengland.org](http://www.rarenewengland.org)

**Rare New England**  
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With Rare and Complex Disorders 


**Rare Share for Rare Disease Day Awareness**

As a part of our Rare Disease Day awareness program RNE is inviting patients, caregivers and health care professionals to submit their stories about living and/or working with rare diseases. We will be highlighting these stories through social media, emailing, our website and compiling for future awareness projects beginning February 1st and leading up to Rare Disease Day on February 28th. We would like to share stories from patients - children and adults, caregivers of all types, and also from those treating these patients and involved in the rare disease community. Let's hear from those touched by different diseases; this is a chance to name your rare diseases and spread awareness of your own particular struggles and share positive experiences as well that will potentially help others.



**Criteria:**

- 350 word limit
- Patient/caregiver/health care professional living and/or working with rare diseases
- Rare disease name and brief summary
- Picture
- Personal characteristic of patient (to emphasize patient vs disease)
- Present an obstacle
- Present a positive experience



Please submit your story to [info@rarenewengland.org](mailto:info@rarenewengland.org) ASAP!

## TESTIMONIALS

"RNE come into my family's life just when we needed the organization the most. Our sons recently were given the Mitochondrial Disease diagnosis when this organization began. The information and the support of meeting others on similar journeys was and is much appreciated. Julie Gortze has been a phenomenal host and organizer. Her knowledge and the speakers she has arranged have been extremely helpful. MNE has been life changing and we are grateful". -Anne

"I discovered Rare New England last summer, a few months after receiving my son's diagnosis. This was during a time that I had so many unanswered questions and, like with most rare diseases, very little resources to look to. [RNE] was there for me though. Julie Gortze helped connect me with other families, doctors, and resources and information that has made this journey much more bearable. [RNE] offers valuable and irreplaceable resources for families in our area and I will be forever grateful". - Laura

"I'm a teen living with several rare diagnoses. About a year ago I attended a game night in Massachusetts for teens living with rare and complex illnesses. The event made a lasting impact on me, and I have several friends I made that I've kept in touch with. Over the past year they've served as someone I could count on to support me during all of the highs and lows life's thrown at me. I also wanted to thank you, although I know it's late, for that event which I think helped me to form some much needed, potentially life-long friendships. - KS

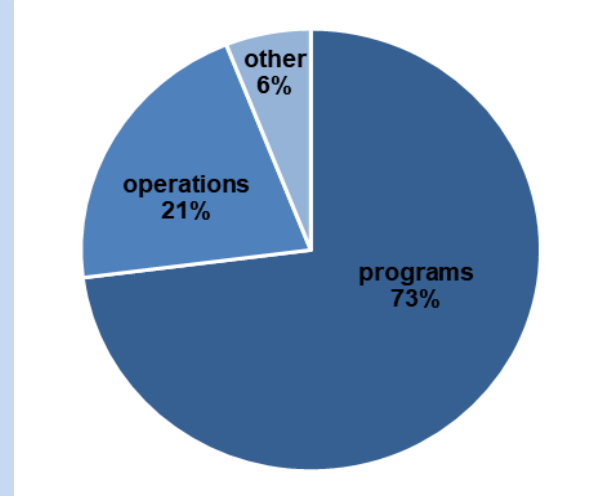
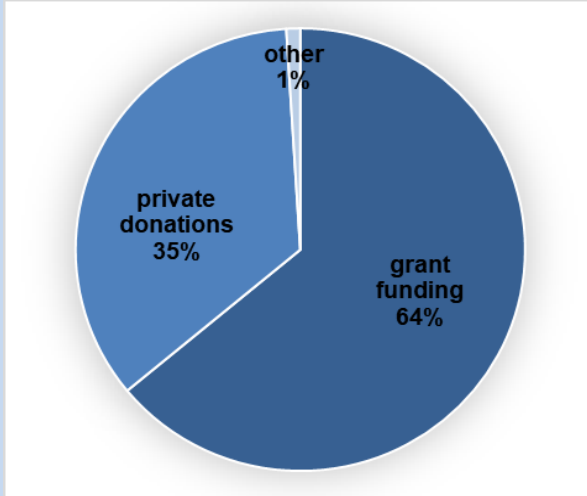
"As a patient advocate who works with rare diseases, an important part of my job is connecting families to local and regional resources. Rare New England has been a wonderful and dedicated resource for rare disease patients and families for several years, and I never hesitate to send my families their way! RNE works hard to provide workshops and information sessions, often featuring local physicians and clinics, helping patients and families explore the latest care information, and making them aware of local medical options and resources. I also appreciate that RNE works collaboratively with other rare disease organizations in New England and around the nation. This gives patients and families an easy way to connect with other medically complex families in their communities, and increases the advocacy and awareness power of all rare disease patients throughout the region. RNE is a fantastic group, and a great resource for the New England area." - Jessica Shriver

"I am just writing to say how much I appreciate that you created a section on your website all about special needs and people with disabilities. I have a special needs brother and have a blog for families with children with autism and I know how much this extra effort means to families with special needs kids.

It was extremely thoughtful of you to share this information for families in need". - Linda

## FINANCIALS

Rare New England's revenue for 2017 was \$12,464. Revenue came from grants, private donations, and other sources. 64 percent of revenue came from grant funding, 36 percent came from private donations, and other. Expenses for 2017 totaled \$9,724. 73 percent of funds were spent on programs and events, 18 percent of funds were spent on operations and the rest was allocated to the other category. In 2016, total revenue for Rare New England was \$12,920 and expenses totaled to \$11,346. Revenue decreased by \$455 and expenses decreased by over \$1,600.





## AMPLE THANKS

The successful year of 2017, could not have been possible without the support and dedication of the Board of Directors as well as Rare New England Committee Members and Team Volunteers. Rare New England would like to thank everyone who has

contributed to RNE's success **patients and by-one**, whether financial shared insights, suggestions, time projects, and for support for our disease

RNE appreciates collaborations organizations, medical businesses who have all had a part in new patient organization. Each one has shown remarkable understanding and passion for the rare disease cause and RNE has learned a great deal more by working with them.

Rare New England would also like to sincerely thank their donors - Shire, Sanofi Genzyme, Gene DX, Invitae, Harvard Pilgrim, City Spirits, Shaw's, and Stop & Shop, as well as all our individual donors who have made our services possible .



contributed in **assisting families one-through** contributions, feedback and spent in our providing us work in the rare community.

the with other facilities, and our success as a

## CALL TO ACTION



Rare diseases impact 350 million people worldwide; more than both cancer and HIV/AIDS combined. These diseases might be incredibly specific and individual on their own, but the mark that they make on society is enormous. Sadly, the amount of attention that these unique diseases get is very limited and quite unremarkable.

According to the 2013 census, there were 14,618,806 people living in the New England area, 1 out of every 10 people affected by rare disease. Approximately 50% are children, 30% of whom will not live to see their 5th birthday. (Global Genes)

Many rare disease patients will wait years to find their correct diagnosis, receiving multiple incorrect diagnoses and visiting many different specialists in the meantime. Many rare disease patients suffer with reduced qualities of life due to large medical expenses, difficulty finding treatment and medical care, lack of education and awareness about their disease, feelings of isolation and worry, loss of jobs and careers, long term disability and even more.

To have a lasting impact on rare diseases and the people that it affects, we need to act. The first step towards this fight is promoting awareness. To combat an issue, it first must be understood and the public needs to see it as an issue. As it stands right now, there are many people in the world that do not even consider this a problem. Only those that are affected by it themselves, or those that have a close relationship with someone afflicted knows just how much of a problem it is. There are even people that do not consider some of these diseases to be real, let alone a problem. Therefore, Rare New England strives to raise awareness and promote research in addition to treating individuals and their families.

Facing a problem is like starting a marathon; it all begins with a single step. The first step in our marathon is raising awareness for rare and complex diseases and showing the rest of the world, just how much harm comes from it. We don't know what will need to happen after that, but we will never know if we don't try to promote the awareness that this problem needs. The first step is action, after that the pieces will fall into place.



**PHOTOS**

**Exercise and Physical Therapy with a Rare Disorders**

**Dr. Michael Healy spoke about Connective Disorders**





## CONCLUSION

As a nonprofit, we are proud to report our growth and success during 2017. Rare New England will continue to grow and develop over the years in hopes of combating the increasing need for assistance with rare conditions in the area. Despite the long road ahead of us, there was still progress, and many milestones were met. Rare New England was able to reach these milestones because of the amount of support that was received, and we are eager to grow and develop as our support increases. We welcome the new year and look forward to continuing to carry out our mission of bringing together New England patients, families and providers touched by rare and complex disorders.

