



amyloidosis
research
consortium

Annual Report 2015





“Building on the ARC’s remarkable start, I believe we can change the future for amyloidosis patients”

Isabelle Lousada

CEO and President of
Amyloidosis Research Consortium



Dear Friends

2015 was a ground-breaking year with the launch of the Amyloidosis Research Consortium (ARC). Thanks to your generous support the ARC has rapidly become a force in speeding and advancing the development of much needed treatment for patients with all types of systemic amyloidosis.

Patients are at the very heart of everything we do; their needs drive every decision we make. With promising novel treatments on the horizon, there has never been a more important time in the history of amyloidosis to come together to drive research forward. We are at a critical point. No single individual or organization will succeed alone.

Our partnerships across all stakeholder groups are creating an unparalleled energy and enthusiasm resulting in unrivaled programs. We bring together the best scientists in academia and the pharmaceutical/biotech industry to ensure that promising ideas get attention. The ARC collaborative network focuses on analytic tools to accelerate delivery of new treatments to the clinic.

The ARC has become the driving force in establishing new paradigms and frameworks to accelerate drug development in amyloidosis. This report highlights our achievements, plans and the value this delivers for patients.

None of our achievements or plans would have been possible without the tremendous support, generosity, courage and commitment of patients. The urgent need to develop treatments drives us tirelessly forward. ARC is committed to building on the momentum created in our inaugural year, and with your continued support we will journey forward to reach our goals of developing the right treatment for each patient.

Sincerely,

Isabelle Lousada



ARC 2015 Highlights

Educating Physicians at Scientific Meetings

- Scientific meetings were held at the American Cardiology Conference and American Society of Hematology (ASH) to identify and address challenges in diagnosing amyloidosis.
- ARC provided support for the first Satellite Symposium at ASH devoted entirely to diagnosis and treatment of amyloidosis.

Creating a Collaborative Network

The ARC Collaborative Network brings together experts to develop and share assets across institutions, and build a platform for discovery and advancement of the very best science.

- The founding centers include six leading centers in the US, UK and Italy with a commitment to partnering to advance science.

Advocating for Amyloidosis

The ARC took patients and caregivers to meetings and lobby days in Washington, DC to discuss policy issues affecting amyloidosis patients. The ARC team advocates for patient needs at all stages of the drug development continuum with companies, experts and government.

Conducting Vital Research

- **Biomarkers:** Initiated study with experts to validate critical biomarkers that will lead to earlier diagnosis, improvement of treatments and acceleration of trials.
- **Patient Study:** This widely published study identified key areas that directly affect the patient experience and chance for best outcomes, and the development of programs to improve diagnosis and treatment options.

Driving Patient Focused Drug Development

At the landmark **Patient Focused Drug Development Meeting** patients shared their stories with the FDA, providing valuable data on acceptable benefit versus risks for development and approval of treatments for amyloidosis. The meeting was the first of its kind, and heralded as a model for other disease foundations to follow.

“I commend the Consortium, I think their strategy is the only real way to get things done effectively in a rare disease.”

Janet Woodcock
Director CDER, FDA





Future Directions

Expanding the Collaborative Network

As the ARC grows in 2016 the Collaborative Network plans to expand to include 20 of the world's leading amyloidosis centers.

Driving Research

ARC will support the best research, and build a prioritized portfolio of studies to advance the science and understanding of amyloidosis. Projects currently in development include collecting and analyzing data to support the validation of biomarkers and collaborating with our investigators on promising clinical trials.

Building Awareness

ARC's programs are continually expanding with the development of a series of physician tools including an interactive app. ARC will continue to meet with all levels of government, industry and science to ensure policies and programs reflect the real needs of amyloidosis patients.

“ARC has given incredible visibility to our rare disease. I feel real hope for the future.”

Dawn Gimbel-Myers
ATTR Amyloidosis Patient



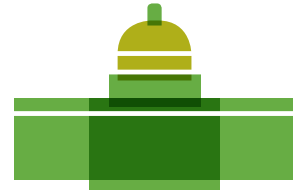


ARC 2015 Landmarks



240

Attendees at
FDA Meeting



29

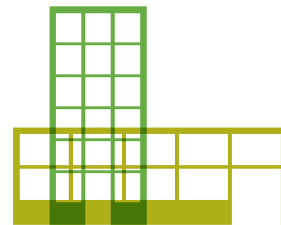
Legislative Meetings



3

International
Scientific
Conferences

6



Founding Centers in
Collaborative Network



7

Key Publications
including Journal Articles
and White Papers



533

Patients Participated
in ARC Study

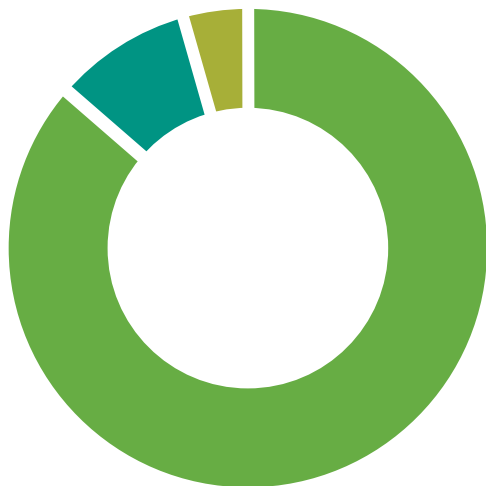


ARC 2015 Statement of Activities



ARC 2015 Source of Funds

- Contributions
- Private Company Grants
- Foundation Grants
- In kind contributions



ARC 2015 Spending Allocation

- Programs
- Administrative
- Fundraising

Support and Revenue 2015

Contributions	\$	205,981
Private Company Grants		344,000
Foundation Grants		200,000
In-kind contribution		127,900
Interest		41

Total Support and Revenue 877,922

Expenses

Programs	
Research/Awareness/Education	373,955
Supporting Services	
Administrative	41,504
Fundraising	18,423

Total Expenses 433,882

Change in net assets 444,040

Net assets, beginning of year 0

Net assets, end of year 444,040

** The amounts are derived from the audited financial statements. The full audit is available upon request.*



“2015 has been a year of great beginnings for the ARC. Many current and future lives hang in the balance. The ARC is creating a platform that is changing the landscape of the disease.”



Raymond Comenzo
Board Co-Chair of Amyloidosis Research Consortium

Thank you

Philanthropy fuels every aspect of ARC’s mission. The ARC is bringing amyloidosis out of the shadows and into the forefront of medical innovation. It is the world’s only nonprofit organization solely dedicated to the accelerating the development of amyloidosis specific treatments and improvement of outcomes.

Every step we take towards our goal of helping amyloidosis patients live longer and with a better quality of life is only made possible thanks to our incredible supporters. We are very grateful to everyone who donated or helped to raise funds for the ARC in 2015.

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**amyloidosis
research
consortium**

**Accelerating the
development of
advanced diagnostic
tools and effective
treatments for systemic
amyloidosis through
collaboration
and innovation.**

Amyloidosis Research Consortium, Inc.
275 Grove Street
Suite 2-400
Newton, MA 02466

e: arc@arci.org
t: 1-617-899-8810
w: arci.org