amyloidosis
research
consortium

Annual Report 2016

"I am very optimistic not only for what innovation lies ahead but also for the opportunity to transform the culture of research to one driven by the needs of amyloidosis patients."

Isabelle Lousada
CEO and President, Amyloidosis Research Consortium

Dear Friends

2016 has been an exciting year for the Amyloidosis Research Consortium (ARC). In the short time since our launch a year ago, we have taken massive strides to become the leading non-profit organization exclusively focused on accelerating research in amyloidosis.

With an increasing interest by pharmaceutical companies in the amyloidosis diseases, there has never been a greater need for a patient focused organization to play a significant role in informing the development of much needed treatments and ensuring they are accessible to patients.

ARC has been at the forefront of bringing together experts from different fields who have, up until now, worked in isolation. I am both excited and inspired to see that our core belief in the need for collaboration and strategic partnerships across stakeholder groups is breaking through the barriers that slow research.

This collaboration while working closely alongside patients allows their voices to be heard and informs research, drug development and overall patient care.

We are proud to share with you our key achievements from the past year which are included in this report. These could only have been realized through the generous support of friends like you. We continue to set ambitious goals to scale ARC's impact in 2017. Only together can we make the strides to transform the lives of those affected by amyloidosis.

Sincerely,

Isabelle Lousada

ARC 2016 Highlights

Raising Awareness and Patient Advocacy

Members of ARC's leadership team spoke at numerous events in 2016, highlighting the important role and achievements of ARC and their position as thought leaders in the rare disease field; including:

- Rare Disease Congressional Caucus briefing
- International Symposium on Amyloidosis
- Listening Session with the commissioner of FDA
- Global Genes Summit

ARC held key meetings that brought together industry partners, patient representatives and experts to facilitate sharing resources and, as a result, ARC was able to develop a comprehensive set of tools and programs to have a far-reaching effect for earlier diagnosis.

ARC and their committed patient advocates played a critical part in the advocacy for the 21st Century Cures Act, which came into law in December 2016. This Act will help accelerate the discovery, development and delivery of cures for diseases like amyloidosis.

Conducting Vital Research

Throughout 2016 ARC has been developing a prioritized portfolio of key research studies in AL and ATTR amyloidosis. These studies focus on gaining a better understanding of the diseases and accelerating development of novel treatments for these diseases with such a great unmet need.

Biomarkers are crucial to accelerate drug development. ARC has done much work in this area, including publishing a landmark paper concluding that a biomarker predictive of survival for patients with AL amyloidosis, should be accepted as a surrogate end point for survival in pivotal clinical trials to greatly facilitate the development of targeted therapeutics. We continue to work on identifying and validating biomarkers specific to different amyloid diseases.

Educational Programs for Earlier Diagnosis

ARC supported a Satellite Symposium at the Annual Meeting of the Heart Failure Society of America with a panel of highly distinguished speakers.

ARC created an education resources section on their website which provides expert-created continuing medical education (CME) courses available for free throughout the year.

Scaling the Collaborative Network

An additional 19 leading amyloidosis centers across 4 continents have joined ARC's Collaborative Network in 2016 to accelerate drug development and clinical trials in amyloidosis. A total of 25 centers of excellence are now members of the network. By implementing a dynamic team approach when working with stakeholders and regulatory bodies, this network creates a unique research platform to conduct ARC studies.

Building a Relationship with the FDA

ARC has been in close collaboration with the Food and Drug Administration (FDA) and, at their request, produced two vital documents: The Patient Voice and Guidance for Drug Development in AL Amyloidosis. These key documents provide a clear pathway for successful patient focused drug development in amyloidosis. ARC was the first organization to write and submit a document of this kind, and is charting a course for other rare diseases.

Diagnostic Tools

The Amyloidosis Clinical Resources App was developed to provide critical clinical disease information for health care professionals. This valuable resource provides a wealth of information and has been widely downloaded and used by both treating and diagnosing physicians.

Future Directions

As ARC moves into 2017, and our third year of operation, we will continue to grow as an organization and embrace the opportunities ahead of us. We have an ambitious and innovative plan in place to systematically address and remove the barriers that are either preventing or slowing down research, the development and access to new effective treatments for amyloidosis.

The research platform and partnerships created by the Consortium will enable the development and approval of exciting new treatments. \We will continue to drive forward programs that will change amyloidosis into easily diagnosed and treatable diseases.

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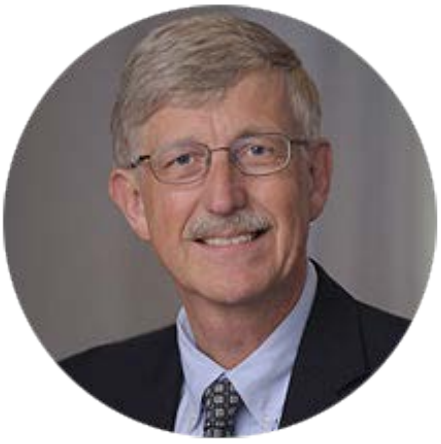
"With all of the exceptional opportunities facing biomedical research today, partnerships and team science are more important than ever."



Francis Collins, M.D., Ph.D.
Director, National Institutes of Health

ARC Model

Our research model is focused on developing the critical tools, assets and infrastructure needed to accelerate progress.

Regulatory Programs

The ARC has an exceptional relationship with the FDA and other regulatory bodies, developing programs to support the review of new treatments and to ensure patient needs are well understood and fully integrated into the review process.

Scientific Discovery

We use cutting-edge science to identify and test new treatments. For example, our work on biomarkers is helping to improve the specificity and success rate of early stage research. This can help to identify drug candidates that work in specific patient groups, therefore accelerating progress.

Making a significant and material contribution to the curability of systemic amyloidosis diseases.

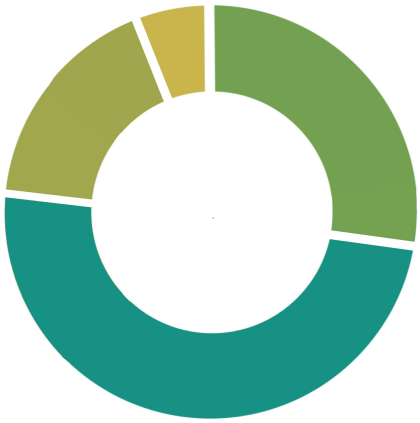
Patient Programs

Patients who are informed and supported are able to make better decisions about their treatment and care. Our programs have been developed to support patients from time of diagnosis to finding an amyloidosis specialist and continues on to help them participate in research studies. ARC also works closely with patients to ensure their preferences and views on the value of products are well understood.

Physician Programs

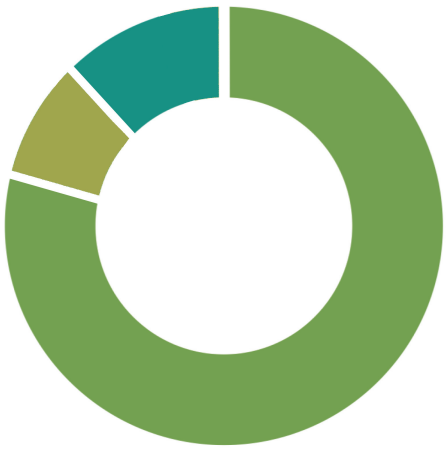
The ARC's awareness and educational programs are extensive and include symposia, round tables and awareness campaigns, as well as ARC publications on disease guidelines and educational tools.

ARC 2016 Statement of Activities



ARC 2016 Source of Funds

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



ARC 2016 Spending Allocation

- Programs
- Administrative
- Fundraising

Support and Revenue		2016
Contributions	$	233,835
Private Company Grants		427,070
Foundation Grants		150,000
In-kind contribution		49,500
Interest		164
Total Support and Revenue		**860,569**
Expenses		
Programs		
Research/Awareness/Education		562,830
Supporting Services		
Administrative		81,919
Fundraising		63,391
Total Expenses		**708,140**
Change in net assets		**152,429**
Net assets, beginning of year		**444,040**
Net assets, end of year		**596,469**

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

“It is humbling to know that ARC is fighting not only for our quality of life, but for the chance to live. Their support provides hope beyond measures.”

Christina Lindsey
Amyloidosis Patient

Thank you

Philanthropy fuels every aspect of ARC’s mission. 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 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 ARC in 2016.

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

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