



Amyloidosis
Research
Consortium

ANNUAL REPORT

2017

ARCI.ORG



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MISSION

Our mission is to accelerate development of and access to new and innovative treatments for all types of systemic amyloidosis.

We work in partnership with researchers, clinicians, industry, regulators and patients to accelerate research and the discovery of new treatments.

We support a bench to bedside and back again approach translating scientific findings into treatments for patients and increasing the understanding of the disease process, and in turn learning from patient experience to refine and direct the next generation of research.

We were founded by a patient; we understand patients' needs and can focus on what will bring significant improvements to the quality of their lives.



LETTER FROM THE PRESIDENT

Dear Friends,

Thanks to your support, 2017 was a tremendous year for the Amyloidosis Research Consortium (ARC) and the patients and families we serve.

2017 stands out as one of the most significant years in the landscape of amyloidosis—two novel agents for hereditary ATTR amyloidosis demonstrated efficacy in clinical trials—these breakthroughs will significantly change the outlook for patients.

With a rising interest in amyloidosis, ARC has continued to grow and play a key role in accelerating research towards a cure.

With the ongoing support of our incredible community and partners, I am confident that we will continue to see significant breakthroughs and new treatments that will transform the lives of patients living with amyloidosis.

We could not do it without the generosity of patients, their families, friends and our partners. We are honored by the trust you have placed in ARC to accelerate the research that will have the greatest impact on patients' lives.

Sincerely,

A handwritten signature in dark blue ink that reads "Isabelle Lousada".

Isabelle Lousada



OUR MODEL

We are focused on developing the critical research tools, assets and infrastructure needed to accelerate progress in amyloidosis research.

Our research model is predicated on speed, efficiency and excellence. We have a razor-sharp focus on delivering outcomes for patients within an acceptable timeframe.

ARC builds collaborations across industry, academia, regulatory and other relevant stakeholders to align research strategies, ensuring that scarce research resources are optimized and directed to prioritized areas of research.

Ultimately, our work is dependent on the successful approval, adoption and diffusion of new treatments and diagnostic tests in health systems across the world. That is why ARC is also focused on understanding the unmet needs and the value of treatments to patients to ensure market access across the world.



Our Key Areas of Focus



ACCELERATING DIAGNOSIS

Building comprehensive programs to educate physicians and provide the tools needed to ensure that patients are diagnosed earlier.



ADVANCING RESEARCH

Actively collaborating with key stakeholders to shape the research environment and optimize scarce resources.



ACCESSIBLE TREATMENT

Engaging with policy makers to improve the understanding of amyloidosis and to speed new therapies to market.



SUPPORTING PATIENTS

Empowering patients with innovative educational tools and support to ensure they have access to the best quality of care.



2017 HIGHLIGHTS

Research

ARC has a dedicated research team focused on advancing a prioritized portfolio of research. Our research is focused on understanding disease progression, identifying biomarkers, and new clinical endpoints to accelerate drug development.

Biomarker Development

ARC has done significant work over the past few years with researchers and the FDA to pursue the validation of a biomarker --an indirect measure of the disease--for clinical trials, an example of which would be using NT-proBNP as an indicator of cardiac health in AL amyloidosis.

Patient Panels

As part of ARC's role in supporting patient focused drug development, we have established a very successful patient panel program. These panels give pharmaceutical companies input into trial design, and other key areas.

Industry Advisory Council

Members include the ten pharmaceutical companies developing products for amyloidosis. ARC provides a valuable platform to create cross company partnerships in the pre-competitive space.

Regulatory

Thanks to its close partnership with the FDA, ARC has become a leading voice in the rare disease field. We have taken part in leadership forums and panels to develop guidance for patient focused drug development, ensuring that the patient voice is included in all discussions.

Awareness and Education

We presented at 12 Conferences and meetings; hosted two educational round tables, and one satellite symposium at the American Heart Failure Society of America's annual meeting (HFSA) with a 12 month enduring online course in Continuing Medical Education.

Cardiac Study

ARC conducted a study on the journey to diagnosis for cardiac amyloidosis patients, with a focus on identifying barriers and solutions to accelerate diagnosis. The results were shared at major medical conferences.

Patient Programs

My Amyloidosis Pathfinder (MAP) is an interactive tool that helps patients find treatment centers and matches them to clinical trials. This bespoke tool was developed in conjunction with Patient Discovery who provided the platform, programming and design components. Patients are highly engaged, and actively being matched to clinical trials, and alerted of new trials.



HOW TO HELP

What will your legacy be?

Planned gifts have a profound and lasting impact on the field of research.

Consider naming ARC as a beneficiary of your:

- » Will or Trust
- » IRA or other retirement plan
- » Life Insurance policy
- » Charitable Gift, Annuity, Remainder Trust, or Lead Trust

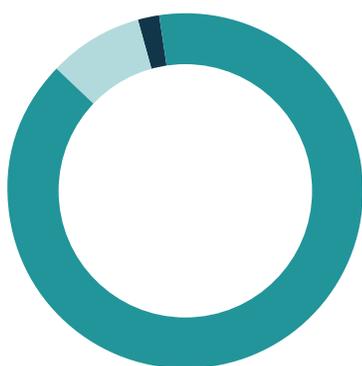
Your support will help pursue innovative research that can ensure better quality of life for patients diagnosed with systemic amyloidosis and bring us closer to a cure.

Please contact us to discuss at arc@arci.org or 617-467-5170.



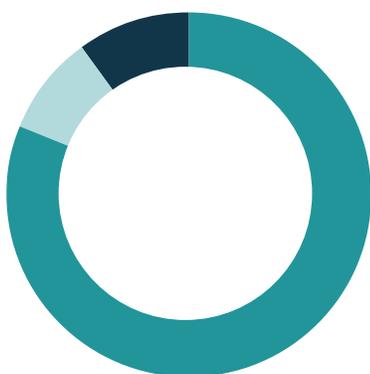
FINANCIALS

ARC 2017 Statement of Activities



ARC 2017 Source of Funds

- Contributions
- Foundation Grants
- In-Kind Contributions



ARC 2017 Spending Allocation

- Programs
- Administrative
- Fundraising

Support and Revenue	2017
Contributions	\$ 1,177,697
Foundation Grants	100,000
In-kind contribution	13,504
Interest	313

Total Support and Revenue	1,291,514
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Expenses	
Programs	
Research/Awareness/Education	742,504
Supporting Services	
Administrative	83,893
Fundraising	92,267

Total Expenses	918,664
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Change in net assets	372,850
Net assets, beginning of year	596,469
Net assets, end of year	969,319

* The amounts are derived from the audited financial statements.

The full audit is available upon request.



LEADERSHIP

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 2017.

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Amyloidosis
Research
Consortium

Accelerating the
development of and
access to new and
innovative treatments
for amyloidosis.

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