Amyloidosis Research Consortium

ANNUAL REPORT

2018

ARCI.ORG

CONTENTS

OUR MISSION

The Amyloidosis Research Consortium is a nonprofit accelerating the development of and access to new and innovative treatments. We are driving the research that will have the greatest impact on length and quality of life for patients.

LETTER FROM THE PRESIDENT

Dear ARC Supporter,

It is my pleasure to share with you ARC's accomplishments of the last two years. While this is formally the 2018 Annual Report, I also wanted to update you on the achievements of 2019.

Overall, I could not be more pleased with the advances of the last two years, not only for ARC but for the entire amyloidosis community. The approvals in 2018 and 2019 of the first treatments for ATTR amyloidosis marked a milestone in our collective experience and brought hope to many. For ARC, it deepened our resolve to continue to forge ahead towards our goals of making a significant contribution to increasing survival in all types of amyloidosis, and measurably improving patients' quality of life.

On pages 8-9, you will see a timeline that depicts many of our major accomplishments since our founding in 2015 through 2018. I will not go over each of these important events individually, but I would like to touch on a few of these specifically, given both their importance to our progress and their demonstration of ARC's commitment to creating partnerships amongst all our stakeholders, from patients to researchers to industry to regulatory. It is these collaborations – consortia, really - that will drive the next stage of research and drug development that will benefit ever more amyloidosis patients.

We began 2018 with a seminal event: a gathering of twenty amyloidosis experts for a research roundtable that produced a summary document of research priorities and critical success factors entitled Advancing Amyloidosis, A Research Roadmap, which you can view on our website at www.arci.org/resource/research_roadmap/. This roadmap has driven much of our work for the last two years, as well as providing guidance to others working to improve the diagnosis and treatment of amyloidosis. Perhaps most importantly, this meeting exemplifies all that ARC is about: bringing together experts in the field to advance research that will most benefit patients, and making that information available to all.

The focus of our own research and other programs, is driven in large part by the priorities outlined by the Research Roundtable. Over the course of 2018 and 2019 we presented posters at several medical and professional conferences summarizing research that was

conducted and led by ARC. Topics included results from ARC's survey of cardiac amyloidosis patient and their caregivers, presented at the 2018 International Society of Amyloidosis (ISA) and the 2018 American College of Cardiology meetings.

An ARC-led collaboration similarly was behind the publication in September 2019 Guidelines for the diagnosis of cardiac ATTR amyloidosis in the journal of the American Heart Association, Circulation: Heart Failure. ARC looks forward to the future publication of similar consensus reviews focused on neurological, and other more general symptomatology of ATTR amyloidosis. Earlier diagnosis leads to earlier treatment, and is critically important in leading to improved outcomes for amyloidosis patients.

We also have several research projects underway, both ARC-led, and in collaboration with other groups. Presently we are working to develop data standards and a virtual biobank, which we believe will be of great benefit to both researchers and amyloidosis centers and will encourage the collaborative efforts that are so important to the progress of science. Particularly in a rare disease, the ability to standardize and make available data is crucial to the advancement of research and the development of new diagnostics and therapies, In addition, as yet another example of our patient-centric focus, we have begun a research project focused on creating a patient-reported outcomes measurement tool in ATTR amyloidosis that will be used to understand and measure the impact of the disease on patients from the patient's perspective. As always, we continue to emphasize the importance of quality of life for amyloidosis patients, throughout their journey with amyloidosis.

Perhaps the most exciting example of our commitment to open collaboration, however, took place on November 12, 2019, when the inaugural Amyloidosis Forum was convened at the U.S. Food and Drug Administration. (FDA). The meeting, a result of the establishment of our Private Public Partnership with FDA, was a resounding success, with over 100 attendees filling the Great Room at FDA's White Oak campus.

The panel of leading experts in amyloidosis was complemented by delegates of seven divisions of FDA, industry representatives and patient speakers. The topic of the meeting was "Advancing Drug Development in AL Amyloidosis" and engendered conversations that will lead to a publication and several more meetings more specifically focused on addressing the challenges in drug development for amyloidosis. We are extremely excited about the success of this meeting and are looking forward to the next Forum that is scheduled for the spring of 2020.

Personally, I was honored to be among a small group named as a 2018 RARE Champion of Hope by Global Genes. This award, which recognizes individuals for their extraordinary achievements in the field of rare diseases, was established by Global Genes, a leading

patient advocacy organization for rare and genetic disorders. In addition, I was named as an inaugural member of FDA's Patient Engagement Collaborative (PEC), which was formed in the summer of 2018. PEC is comprised of external patient community stakeholders who can offer their experiences and perspectives on patient engagement in FDA's regulatory processes. This new collaborative effort was facilitated by provisions in both the 21st Century Cures Act of 2016 and the Food and Drug Administration Reauthorization Act of 2017, which aim to foster patient participation and incorporate patient experiences in the regulatory process.

As I hope you can tell from the brief summary above, and from the chart on the following pages, ARC's determination to make a significant impact on the curability of amyloidosis is being realized in many ways. We will continue with our work, never losing sight of what is most important: amyloidosis patients and their families. Our commitment to improving the length and quality of their lives is unwavering and I look forward to keeping you updated as to our progress.

Sincerely,

Isabelle Lousada

WHAT WE DO

- We enable pioneering research to better understand the factors responsible for the onset and progression of amyloidosis
- We accelerate the discovery and development of new and effective treatments and support their approval, reimbursement, adoption and diffusion into health systems across the world
- We raise awareness and provide education for physicians about the signs, symptoms and care of amyloidosis to raise the level of suspicion, improve the speed of diagnosis, and standards of care
- We provide information and support for patients and their families to help them cope with everything an amyloidosis diagnosis brings and to empower them to make informed decisions about treatment and care

WE WORK BY

- Optimizing scarce resources
- Creating new synergies and dynamic partnerships across all stakeholder groups
- Prioritizing the research most likely to have the greatest positive impact on patients
- Advocating for more funding to be directed toward the most promising areas of amyloidosis research

THE UNMET NEED

- Only 30% of AL patients report a definite improvement after receiving treatment
- 35% of patients receive inaccurate treatment due to a misdiagnosis
- 29% of hATTR patients report the disease greatly impacts emotional well-being
- 49% of patients have little to no access to relevant clinical trial information
- 73% of patients are unsure how to enroll in a clinical trial

4 or more
average number of doctors seen before diagnosed

1.6 million
African Americans carry the TTR V122I genetic mutation at risk of developing ATTR Cardiac Amyloidosis

150 miles
distance from an amyloidosis center for majority of patients

50%
or more of AL patients have clinically significant heart involvement at diagnosis[1]

Experts believe wildtype amyloidosis may not be a rare disease[2]

40%
may not benefit from the available treatments for hATTR[3]

3,000
patients diagnosed in the US each year with AL amyloidosis[4]

25%
of wild-type ATTR patients have symptoms for >4 years before being diagnosed

10 YEARS, $2.6 BILLION
to develop, test, and bring a new drug to market[6]

1. Orphanet J Rare Dis. 2012; 7: 54. Published online 2012 Aug 21. doi: 10.1186/1750-1172-7-54
2. JACC Heart Fail. 2014. Apr;2(2):113-22. doi:10.1016/jchf.2013.11.004. JACC Sep 2016, 68(10) 1021-1023; DOI 10.1016/j.jacc.2016.06.032
3. N Engl J Med. 2018 Jul 5;379(1):82-85. doi: 10.1056/NEJMe1805499.
4. Incidence and natural history of primary systemic amyloidosis in Olmsted County, Minnesota, 1950 through 1989.Kyle RA, et al. Blood. 1992 Apr 1; 79(7):1817-22.
5. https://www.phrma.org/report/biopharmaceutical-research-and-development-the-process-behind-new-medicines

ARC'S IMPACT

ARC has grown rapidly since our inception in 2015, and has become a leading organization in the field of rare disease research.

ARC has built collaborations across industry, academia, and regulatory agencies, to align research strategies and create a clear pathway for drug development and early diagnosis. Through our innovative programs we have brought patients to the forefront of amyloidosis research. This integral partnership with those affected most by the disease has increased the global understanding on what matters to patients and has significantly reshaped and enriched the research environment.

PROGRESS TO DATE

2015

FDA DRUG DEVELOPMENT
Our Patient Focused Drug Development Meeting with FDA generated data and reports about patient experiences — this first of its kind meeting was so successful that a pathway has been established for other rare diseases

PATIENT EXPERIENCE STUDY
First of its kind study to define the amyloidosis patient journey and quality of life

2016

ARC RESEARCH ROUNDTABLE
First meeting of researchers, FDA and industry to address challenges of drug development in amyloidosis

PATIENT VOICE
The first Patient Voice publication to be shared with FDA and contain a benefit/risk framework for use in product review

BIOMARKER PUBLICATION
Landmark paper on the importance of the biomarker NT-proBNP in AL amyloidosis

DRUG DEVELOPMENT GUIDELINES FOR INDUSTRY
Drafted extensive guidelines for clinical trials in AL and ATTR amyloidosis

2017

ARC RESEARCH MEETING
Leading experts across academia, industry, regulatory, and other areas identified and published the most pressing needs in research across the drug development continuum, to focus research initiatives

CLINICAL RESOURCES APP
The clinician's app has been downloaded over 2000 times around the world and has been associated with an increase in amyloidosis diagnosis

2018

RESEARCH WHITE PAPER
Consensus document on the most pressing research needs across the entire amyloidosis research and development continuum

MAP TRIAL TOOL
Enabling patients to stay informed about research and clinical trials and connect with amyloidosis treatment centers. Over 1000 patients actively using MAP

PATIENT PREFERENCE STUDY
Conducting a pioneering, patient preference elicitation study to highlight patients' and caregivers' perspectives on potential benefits and risks of treatment options

INTERNATIONAL CARDIAC STUDY
The first international study to understand patients' experience with cardiac amyloidosis

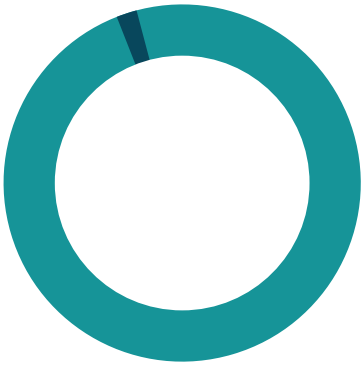
GUIDELINES FOR SUSPICION AND DIAGNOSIS OF ATTR AND WILD TYPE AMYLOIDOSIS
Consensus guidelines to support earlier suspicion and diagnosis of both wild type and hereditary ATTR

DATA SHARING
Promoting and enabling data sharing within the amyloidosis community to advance research efforts and understanding of the disease

COLLABORATIVE PROJECTS
Fostering and supporting collaborative projects to optimize research from the laboratory bench to the patients' bedside

FINANCIALS

ARC 2018 Statement of Activities



ARC 2018 Source of Funds

- Contributions
- In-Kind contributions

ARC 2018 Spending Allocation

- Programs
- Administrative
- Fundraising

Support and Revenue		**2018**
Contributions	$	1,415,847
In-kind contribution		8,000
Interest		597
Total Support and Revenue		**1,424,444**
Expenses		
Programs		
Research/Awareness/Education		1,448,615
Supporting Services		
Administrative		77,658
Fundraising		141,715
Total Expenses		**1,667,988**
Change in net assets		**($243,544)**
Net assets, beginning of year		**969,319**
Net assets, end of year		**725,775**

* 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 2018.

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Amyloidosis Research Consortium

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

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