



Amyloidosis  
Research  
Consortium

ANNUAL REPORT

# 2019

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# A NOTE FROM OUR CEO

The Amyloidosis Research Consortium is dedicated to accelerating the pace of development for new and innovative treatments, and improving quality of life for amyloidosis patients.

Dear ARC Supporter,

Another year, another big step forward for Amyloidosis! Adding to the two drugs approved to treat hereditary ATTR amyloidosis, we saw the approval of tafamidis, (vyndaquel™) the first drug to be approved to treat Cardiac ATTR. We have also seen an increase in the number of pharmaceutical companies engaged in the amyloidosis space. We are proud of the **patient focused** role ARC has played in accelerating the development of new and innovative treatments for amyloidosis that has led to the approval of treatments, with more on the horizon. We will continue to relentlessly push forward in new areas with the goals of making a significant contribution to **increasing survival** in all types of amyloidosis and measurably improving patients' quality of life.

Our focus on patients means that ARC is there for every patient, at each step of their amyloidosis journey, from suspicion of a diagnosis, through finding the best available care, to identifying clinical trial opportunities, and providing education, support and tools helping patients live the best life they can. We are continually expanding our services and programs for patients as well as physicians.



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As we grow our care initiatives, we remain committed to **advancing research**, and as a patient-founded organization, patients' needs are the driver for all that we do in our own work, as well as **driving collaborations** we have built among pharmaceutical companies, researchers, clinicians, payers and regulatory bodies. This was particularly evident with the formation of our Public Private Partnership with the Food and Drug Administration (FDA) and the launch of the Amyloidosis Forum, with the inaugural meeting at the FDA campus in Bethesda, Maryland.

The highlights in this report are a direct result of the collaboration and hard work of the entire community- affected individuals, researchers, pharmaceutical companies, and regulators. **As a patient-founded organization, we know how urgent these goals are. We run our business with the same efficiency that we expect of our partners. We consistently outperform our peers in fiscal responsibility, accountability, and transparency.**

We are so grateful for your continued partnership and support. Together we can and will change the future for all those impacted by amyloidosis.

Sincerely,

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

Isabelle Lousada



# HIGHLIGHTS OF 2019

It is our vision that every patient receives a timely diagnosis and has what they need to treat their amyloidosis.

## **Accelerating Drug Development**

### **Amyloidosis Forum**

ARC and the FDA combined forces creating a Public Private Partnership in the form of the ARC run Amyloidosis Forum, to bring a wide group of stakeholders together to identify and address the barriers that exist in drug development with a particular focus on AL amyloidosis. The Forum was launched in 2019 and has made significant strides to bring this rare disease community together to address the barriers in AL amyloidosis drug development.

Since the initial meeting was held in the Great Hall at the FDA, the Forum has grown from strength to strength and garnered international collaboration from disease experts and regulators alike, and expanded to include EMA and MHRA.

The Forum has formed a number of working groups and convened meetings to focus on key priorities that were identified in the initial Amyloidosis Forum meeting. These priorities include:

- » Novel trial design and endpoint development
- » Natural history
- » Role of Imaging to assess burden of disease/response to therapy
- » Disease specific patient reported outcome measures



## **Improving Care**

### **Masterclass**

For physicians specifically, we have developed the Amyloidosis Masterclass, a Continuing Medical Education (CME) program. The Masterclass is an interactive, educational event where attendees learn about amyloidosis care, the value of multidisciplinary teams, how to build an amyloidosis program to best support patients and connect with experts in the field. These conferences are open to all medical specialties but are primarily targeted to practicing physicians who most commonly see and treat amyloidosis patients.

### **Pathway to Diagnosis**

As far too many patients know from firsthand experience, amyloidosis is challenging to diagnose. Far too many patients experience significant and potentially life-threatening delays before attaining an accurate diagnosis. ARC spearheaded an initiative to convene international amyloidosis experts across all disciplines to develop consensus guidelines. These have resulted in the publication of guidelines for neurologists, cardiologists and general practitioners on the suspicion and diagnosis of ATTR amyloidosis.

## **Putting Patients at the Center of Research**

### **ATTR PRO**

ARC has initiated development of an ATTR PRO (Patient Reported Outcomes) measurement tool to be used to understand and measure the impact of the disease on patients from the patient's perspective. This tool will measure what matters most to patients with ATTR amyloidosis, such as how the disease and/or treatment impact their daily lives, physical functioning, and mental health status. It will allow physicians to monitor patients' progress and can be incorporated into clinical trials as well as daily care for patients.



## Access to Treatments

Regulatory approval is an important step to delivering new treatments for patients, but it is not the final step. Our mission is to ensure that all patients have access to treatments, and our Market Access program has been working on just that.

ARC conducted a patient preference study to generate data that would address the value of novel treatments to the ATTR community. This data was shared with the community along with an ARC Health Technology Assessment toolkit, which we developed to provide a step-by-step guide, along with case studies. It is intended to give information about how treatments are evaluated and approved for use in the various member state healthcare systems and to guide patients and patient groups on how they can participate in the process to ensure those treatments that are developed for their type of amyloidosis can be made available in their countries.

## Patient Programs

ARC conducted a patient survey to evaluate patient experiences. The majority of patients reported feeling overwhelmed when receiving their diagnosis, and only a small percent were given written information or guided to resources to help them come to terms with their diagnosis and learn about their disease. In complex, rare diseases like amyloidosis, patients who are well informed are empowered to be active partners with their healthcare providers to make treatment and care decisions that can greatly improve their quality of life. To support patients throughout their amyloidosis journey ARC has developed the following:

### MAP

The My Amyloidosis Pathfinder (MAP) helps patients evaluate and navigate finding treatment centers, and provides valuable information about clinical trials and research opportunities. With over 2,000 patients engaged and



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regularly using MAP, this tool has become an important way to keep the community informed about research opportunities and accelerate enrollment in clinical trials. Our wonderful support team speak to patients daily and we have seen a significant increase in the number of calls we receive.

## **ARC Talks**

Our webinar series, ARC Talks, delves into a broad range of topics that impact patients, from clinical trials, to treatment options, to managing disease symptoms as well as treatment side effects.

With a rapidly changing treatment landscape, we pride ourselves on having the latest information in a variety of easily understandable formats so that all patients have access to information, and support.





# ADVANCING TREATMENT OPTIONS

The 2019 amyloidosis drug pipeline reflects the amyloidosis community's progress in research.





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# ACKNOWLEDGEMENTS

We would like to acknowledge and thank the **amyloidosis research community** who were involved throughout the development of the Amyloidosis Research Strategy Roadmap.

These include all who attended the 2018 Research Strategy Roundtable meeting to set the prioritization of key themes, from which ARC's 2019 priorities were derived.

We would also like to thank **each and every patient who has taken part in a clinical trial or other forms of research**. We recognize there is no such thing as an easy diagnosis and journey with amyloidosis, and research would not be possible without patients' involvement and the support of caregivers, families, and friends.

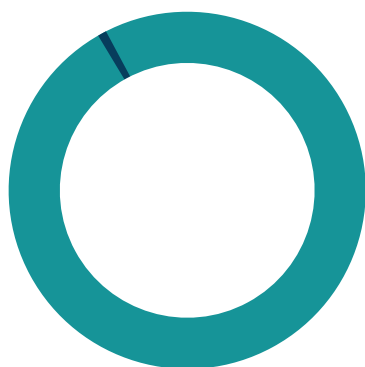
We are grateful to **all the different stakeholders that have collaborated with ARC**, including amyloidosis researchers and clinicians, patients, and patient foundations, pharmaceutical companies, and regulatory bodies, who together play such an important role in bringing new discoveries from lab benches to patients' bedsides.

# THANK YOU



# FINANCIALS

## ARC 2019 Statement of Activities



**ARC 2019 Source of Funds**

- Contributions
- In-Kind contributions



**ARC 2019 Spending Allocation**

- Programs
- Administrative
- Fundraising

<b>Support and Revenue</b>	<b>2019</b>
Contributions	\$ 1,602,333
In-kind contribution	9,300
Interest	857
	.....
<b>Total Support and Revenue</b>	<b>1,706,598</b>
<hr/>	
Expenses	
Programs	
Research/Awareness/Education	1,057,161
Supporting Services	
Administrative	95,702
Fundraising	184,706
	.....
<b>Total Expenses</b>	<b>1,337,569</b>
<hr/>	
<b>Change in net assets</b>	<b>(\$369,229)</b>
<b>Net assets, beginning of year</b>	<b>725,775</b>
<b>Net assets, end of year</b>	<b>1,094,804</b>

\* The amounts are derived from the audited financial statements.

The full audit is available upon request.



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# LEADERSHIP

The ARC board and steering committees are comprised of a diverse group of exceptional individuals dedicated to the ARC mission. Together with the ARC staff, they identify areas of opportunity to inform our programs.

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**Isabelle Lousada** PRESIDENT & CEO

**Kristen Hsu**, EXECUTIVE DIRECTOR OF CLINICAL RESEARCH

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Amyloidosis  
Research  
Consortium

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

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