



# 2023

## ANNUAL REPORT

[ARCI.ORG](https://arci.org)

## 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.

## CONTENTS

<b>Message</b> .....	2
<b>Drug Development</b> .....	3
<b>Programs</b> .....	4
Greater Collaboration .....	5
Better Care .....	6
Supporting Patients .....	7
Increased Support .....	8
<b>Organization</b> .....	9
Thank You .....	10
Financials .....	11
Board & Committees .....	12

# MESSAGE FROM THE EXECUTIVE DIRECTOR

Dear Friends,

2023 marked another transformative year for the Amyloidosis Research Consortium (ARC). Over the past eight years, we have established ourselves as a leading organization committed to advancing the understanding, diagnosis, and treatment of amyloidosis. From a time when no treatments existed for amyloidosis, we have seen the approval of six therapies, and we continue to make strides toward new breakthroughs.

A key milestone this year was the continued success of the Amyloidosis Forum, a pivotal program for driving forward research and collaboration.

By bringing together key stakeholders — including researchers, regulatory bodies, healthcare providers, and patient advocates — the Forum fosters critical dialogue and aligns scientific progress with the real-world needs of patients. The expansion of the Forum to include AL and ATTR amyloidosis further broadens its impact, ensuring that both forms of the disease are addressed comprehensively in ongoing research.

As we reflect on the past year, ARC's dedication to driving research, supporting patients, and engaging stakeholders has remained at the forefront of our

mission. We have enhanced our internal infrastructure, expanded our research efforts, and positioned ARC for even greater success in the future. We are grateful to our partners, donors, patients, and the tireless efforts of our team, all of whom have made our progress possible.



*Isabelle*

**Isabelle Lousada**  
ARC Founder & CEO

Forging the  
**PATH TO A  
CURE**

CURE  
CURE  
CURE  
CURE

# DRUG DEVELOPMENT PIPELINE



<sup>1</sup>Commercial treatments seeking secondary approval

## ADVANCING TREATMENTS

ARC is dedicated to advancing the development of innovative therapies for amyloidosis through strategic collaboration among researchers, clinicians, and industry partners.

By fostering these connections, ARC helps accelerate the progression of promising drug candidates through the pipeline, ensuring that breakthroughs in treatment reach patients more quickly.

# PROGRAMS

ARC programs focus on advancing drug development, improving treatment options for patients, and access to quality care and information



Attendees of the 2023 annual Amyloidosis Forum on June 21.



# Amyloidosis The FORUM



## GREATER COLLABORATION

★ Highlight

### Amyloidosis Forum

The 2023 Amyloidosis Forum continued to build on the progress made in previous years, and expand beyond its sole focus on AL to include ATTR amyloidosis. The Forum fosters collaboration between the Amyloidosis Research Consortium (ARC), the U.S. Food and Drug Administration (FDA), and other key stakeholders in the field. With the support of international partners including the UK's Medicines and Healthcare Products Regulatory Agency (MHRA), the European Medicines Agency (EMA), and Japan's Pharmaceuticals and Medical Devices Agency (PMDA), the Forum expanded its global reach, bringing together experts from across the world to address challenges in drug discovery and development for AL and ATTR amyloidosis.

With the participation of clinicians, patients, drug developers, payors, and regulatory bodies, the Forum aimed to align on approaches to further drug development that would be both scientifically rigorous and meaningful to patients. These efforts were bolstered by continued funding support from the National Institutes of Health (NIH), which recognized the value of the Forum's work in advancing the field of amyloidosis research. Together, these efforts highlight the Forum's commitment to advancing the science of amyloidosis while ensuring that the needs and perspectives of the broader community are incorporated into future initiatives.

# PATIENT FOCUS

ARC is committed to elevating the voices of patients and caregivers to inform research and care. In 2023, we expanded our Community Research Program with two major initiatives designed to better understand the real-world experiences of those living with and affected by amyloidosis. We launched the “Experience and Treatment Preferences of ATTR Patients and their Caregivers” study, providing critical insights that helped shape discussions at the Amyloidosis Forum meeting at the FDA.

At the same time, our Annual Community Survey — translated in six languages — enabled us to gather perspectives from patients and caregivers with all types of amyloidosis across the globe. These collective insights are already influencing research priorities, guiding treatment development, and ensuring that the lived experiences of patients and caregivers are driving progress in care.

# BETTER CARE

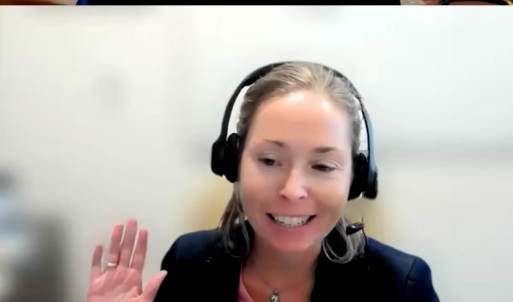
## ★ Highlight

### **Advancing Patient-Centered Care with the ATTR-QOL: A Groundbreaking Tool for Transthyretin Amyloidosis**

ARC’s development of the Transthyretin Amyloidosis Quality of Life Questionnaire (ATTR QOL), a patient-reported outcomes (PRO) measure is a significant advancement in ensuring that research and treatment approaches are truly aligned with the experiences and needs of patients. This innovative tool is the first of its kind, specifically designed to capture the unique experiences of individuals living with all types of ATTR amyloidosis. By enabling patients to report directly on their symptoms and the impact on their daily lives, the ATTR-QOL provides invaluable insights that inform more effective treatment strategies and enhance clinical research. Use of the ATTR-QOL in clinical care fosters a more personalized and patient-centered approach to

treatment, ultimately improving the quality of life for those affected by ATTR amyloidosis.

ARC has partnered with QualityMetric to manage the licensing, scoring, and translation of the ATTR-QOL, which is now available in more than 40 languages. The development and availability of the ATTR-QOL underscores ARC’s commitment to patient-centered care and highlights the vital role of donor support in driving forward initiatives that significantly improve the quality of life for those affected by ATTR amyloidosis.



A few of the hosts from our 2023 ARC TALKS webinars.

# ARC TALKS

WEBINAR SERIES



## SUPPORTING PATIENTS

### ★ Highlight

### Comprehensive Patient Programs: Expanding Resources & Support for the Amyloidosis Community

In 2023, the Amyloidosis Research Consortium (ARC) expanded its comprehensive patient programs to provide even more support and education for those affected by amyloidosis, empowering patients to make informed decisions about their care. The **ARC Talks** webinar series continues to offer expert-led sessions on important topics related to amyloidosis, treatment options, and ongoing research. These webinars provide valuable insights and serve as a platform for patients and caregivers to stay informed and engaged with the latest developments in the field.

ARC has developed a growing collection of disease- and treatment-specific booklets, which are now widely available through amyloidosis centers and ARC.

Our **Trained Support Staff** provide personalized, one-on-one assistance to help patients navigate treatment options, understand clinical trials, and connect with the right clinicians.

Additionally, the **MAP Tool** remains a vital resource, helping patients find the most relevant clinical trials and treatment centers based on their specific needs. Through these expanded programs, ARC is committed to improving the quality of life for those impacted by amyloidosis while advancing research and treatment options.



# CARING FOR THE COMMUNITY

“ARC has been instrumental in providing important information to me as an amyloidosis patient. Thank you for your service. You are making a difference.”

— Amyloidosis Patient

## INCREASED SUPPORT

### ★ Highlight

#### Empowering Patients

ARC's patient support team is always ready to help. Whether reaching out by email, phone call, or online, patients are never alone.

Finding the right support is critical. We're here to help the amyloidosis community as they seek information and face decisions about treatment, ongoing care, and beyond.

This year our team supported 315 different patients requesting information on diagnosis, treatment, helpful resources and emotional support. Through this vital work, we provided clarity, compassion and reliable information to those affected by this disease.

Are you looking for help navigating diagnosis and living with amyloidosis? Our team would love to talk with you about your options:

**W** [arci.org/contact](https://arci.org/contact)  
**T** (617) 467-5170  
**E** [support@arci.org](mailto:support@arci.org)

# ORGANIZATION

Thanks to the hard work and generosity of our supporters, we continued expanding our impact on amyloidosis in 2023. Together we are building towards a future free of amyloidosis.

# THANK YOU TO OUR SUPPORTERS

As we look ahead, the progress we've made at ARC wouldn't be possible without the continued support of our donors and partners.

Your contributions, whether through donations or legacy gifts, directly impact those affected by amyloidosis by advancing research, improving treatment options, and providing essential resources for patients and families. We invite you to join us in this important work, helping us create a brighter future for those living with amyloidosis.

## Together, we can make a lasting difference.

### Give Online

[www.arci.org/donate](http://www.arci.org/donate)

### Give by Mail

Amyloidosis Research Consortium  
320 Nevada Street, Suite 210  
Newton, MA 02460

### Give by Phone

(617) 982-2946



# SUPPORT OUR FUTURE

**Every supporter — whether an individual donor, corporate partner, or foundation — plays a vital role in accelerating research and bringing us closer to a future free of amyloidosis..**

Learn more about ways you can support our mission on our website, including gifts of stock, donor advised funds, individual donations, and more.

# FINANCIALS



## Source of Funds

- Contributions
- Other Funds



## Spending Allocation

- Programs
- Fundraising
- Administrative

## Support Revenue 2023

Contributions	\$ 2,702,741
Investment Income	54,751
Other revenue	130,533
<b>Total Support</b>	<b>2,888,025</b>

## Expenses 2023

Programs	\$ 1,548,608
Administrative	341,679
Fundraising	524,772
<b>Total Expenses</b>	<b>2,415,059</b>

**Net Assets (Start of Year)** \$ 2,215,350

**Net Assets (End of Year)** 2,748,763

**Net Asset Change** 533,413

# BOARD

## **Sarah Cairns-Smith**

*Board Chairman*

Senior Partner & Managing Director, Boston Consulting Group

## **Isabelle Lousada**

Founder and CEO, Amyloidosis Research Consortium

## **Dena Heath**

*Board Secretary*

Facilitator, Northern California Amyloidosis Support Group

## **Jason Shore**

*Board Treasurer*

CEO, Patient Discovery

## **Raymond Comenzo, MD**

Director, Myeloma & Amyloid Program; Tufts University

## **Ashutosh Wechalekar, MD**

Professor of Medicine and Haematology, University College London

## **Rick Nathanson**

Managing Partner, Nathanson and Company

# COMMITTEES

## **Emeritus Board Member**

### **Giampaolo Merlini, MD**

Director, Center for Research and Treatment of Systemic Amyloidosis, Scientific Institute Policlinico San Matteo, University of Pavia, Italy

## **Scientific Advisory Committee**

### **Raymond Comenzo, MD**

Director, Transfusion Services; John C. Davis Myeloma & Amyloid Program; Professor, Tufts University School of Medicine

### **Angela Dispenzieri, MD**

Consultant, Division of Hematology; Serene M. and Frances C. Doring Professor of Medicine and of Laboratory Medicine and Pathology; Hematology Research Chair, Mayo Clinic

### **Mathew Maurer, MD**

Arnold and Arlene Goldstein Professor of Cardiology; Professor of Medicine, New York-Presbyterian Hospital/Columbia University Medical Center

### **Jeffery Kelly, PhD**

Lita Annenberg Hazen Professor of Chemistry, Scripps Research

### **Ashutosh Wechalekar, MD**

Senior lecturer and honorary consultant hematologist, University College London and the Royal Free London NHS Foundation Trust

### **Giampaolo Merlini, MD**

Director, Amyloidosis Research and Treatment Center and Biotechnology Research Laboratories, University of Pavia

### **Vaishali Sanchrawala, MD**

Professor of Medicine; Director, Amyloidosis Center; Director Stem Cell Transplantation Program, Boston University School of Medicine and Boston Medical Center

## **Patient Advisory Committee**

### **Carla Turner**

### **Cordelia Maloney**

### **David Antonaitis**

### **Dawn Gimbel-Myers**

### **Debra Ruehlman**

### **Dena Heath**

### **Desiree Love-Holliday**

### **Eric Eckerstrom**

### **Gloria Neff**

### **James Harrison**

### **Joseph Phillips**

### **Kathleen Burda**

### **Linnie Newman**

### **Michael Jennings**

### **Michael Lane**



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

**Amyloidosis Research Consortium**

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