

More than hope.  
**Progress.**

# PROGRESS REPORT

APRIL 2019

INTERNATIONAL  
PROGRESSIVE MS ALLIANCE

**CONNECT** TO END PROGRESSIVE MS

Kerrie, living with MS  
since 2008, Australia



# TOGETHER, WE ARE STRONGER THAN PROGRESSIVE MS.

The International Progressive MS Alliance is an unprecedented global collaboration of MS organizations, researchers, healthcare professionals, the pharmaceutical industry, companies, trusts, foundations, donors and people affected by progressive MS, working together to address the unmet needs of people with progressive MS. **Our promise is more than hope, it is progress.**

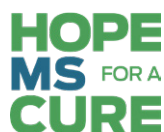
## MANAGING MEMBERS



## MEMBERS



## TRUST AND FOUNDATION MEMBERS



The Al Otaiba Family  
The Alan Buegeleisen Fund  
Anonymous Donor  
The Avidan Family Charitable Trust  
The Chesney Miles Charitable Fund  
Cathy and Bill Onufrychuk  
Dean Smith on behalf of FUMS

Dick and Robin Kelly Foundation  
Foundation for a Better World  
In memory of Georg Bruun on behalf of the Bruun Family and CP ApS  
Margaret and Frank Hofland  
Kathleen C. Moore Foundation  
Rabbits Unlimited Wisconsin  
The Rubschlager Family  
Jim Tidwell MS Memorial Research Fund

M&H Schwartz Family Foundation  
Yellow Rose Gala Foundation

## INDUSTRY FORUM

Biogen  
EMD Serono  
Genentech Roche  
MedDay Pharmaceuticals  
Novartis AG  
Sanofi Genzyme

# ACCELERATING BREAKTHROUGH TREATMENTS

Ending progressive multiple sclerosis is an urgent, unmet worldwide need. Over one million people living with this form of MS face uncertainty, losing ground each day as they experience worsening symptoms.

While we are highly encouraged by the recent approval of three treatments for forms of progressive MS, these are only effective for some. We must seize on the momentum of these early treatments to ensure the rapid development of additional solutions for progressive MS. We must find solutions for everyone affected by MS.

And we must ensure that people with progressive MS can live fulfilling lives—enhancing well-being through symptom management and rehabilitation.

More research. More treatments. More life-changing solutions.

More must be done so that **EVERYONE** has an effective MS treatment.

“ Progressive MS is a complex disease that cannot be solved in isolation. The MS movement is united in identifying effective treatments for progressive MS as quickly as possible. We are a powerful force, but more is needed to sustain momentum and achieve results.

Please join us. Together, we can expand our global movement, remove barriers and accelerate progress for people affected by MS.”

*Cyndi Zagieboylo*



**Cyndi Zagieboylo**  
Alliance Executive  
Committee Chair  
National MS Society  
President & CEO, U.S.

“ While the Alliance’s strategic agenda has had demonstrable impact through expert perspective, international research collaborations, funding and the engagement of people affected by progressive MS, we must be relentless in our efforts. It is critical that the Alliance remains focused on enabling new treatment discovery.

The Alliance has inspired hope and expectation of progress. We must deliver—together, we can deliver faster.”

*Alan J. Thompson*



**Professor Alan J. Thompson, MD**  
Alliance Scientific Steering  
Committee Chair  
University College, London  
Dean of Brain Sciences, U.K.

# A WORLD UNITED IN ACCELERATING SOLUTIONS

## PRIORITIES

- 1 Understand Progression
- 2 Accelerate Clinical Trials
- 3 Enhance Well-Being

Leading MS experts from throughout the world are united in fueling discoveries for new treatments for progressive MS. The Alliance Scientific Steering Committee provides expert perspective and strategic direction. It is a team of researchers,

health professionals and people affected by progressive MS charged with delivering on three priorities necessary for success:

- Understand progression
- Accelerate clinical trials
- Enhance well-being

These vital, inter-connected ambitions are realized through **funding** transformational multi-national research, **convening** scientific summits to rapidly share knowledge and stimulate further discovery, and **collaborating** with regulatory bodies, scientific leaders in other diseases, and with MS pharmaceutical experts through the Progressive MS Alliance Industry Forum.

It is an unparalleled, global effort to understand and treat progressive MS.

# UNDERSTANDING PROGRESSION

## UNLOCKING THE MYSTERY OF PROGRESSION TO IDENTIFY TREATMENT BREAKTHROUGHS

### Two Drug Discovery Networks

#### Protecting and Repairing the Brain



Led by **Professor Gianvito Martino** from Italy in collaboration with investigators from Italy, France, Germany, Canada and the United States, this team is working to identify

agents that may protect nerve cells and/or promote nervous system repair. Through their work, they also expect to better understand what is responsible for causing progressive nerve damage or impaired myelin regeneration—discoveries that would accelerate additional treatment advances.

Using sophisticated software, the team identified over 500 potential drugs to investigate, further analysis and modeling has narrowed the focus to 73 for laboratory testing. The network was the recipient of the 2018 Premio Venezia Prize for the best research enterprise between Canadian and Italian research groups.

“ Professor Martino has established a network of distinguished international experts to implement a highly innovative program, first to identify candidate therapies using a bioinformatic approach, and second to test whether these therapies are effective. The network has achieved impressive progress with both approaches in their first year, making it likely that significant advances will follow.”

– **Professor Raj Kapoor**

U.K., Scientific Steering Committee Member

“ Recent publications in scientific journals Nature and Cell testify to the outstanding productivity of the Quintana network. By unraveling how genes and environment conspire to induce neuroinflammation, this group is in a pole position for discovering new drugs to fight MS.”

– **Professor Reinhard Hohlfeld**

Germany, Scientific Steering  
Committee Member

### Preventing Brain Damage



**Professor Francisco Quintana** from the United States is leading a team of investigators from the U.S., Canada, Israel and drug company Sanofi Genzyme focusing on the innate immune system and working

to identify drug candidates that may be effective therapies in progressive MS. While the innate immune system normally functions to protect the body from infections, Dr. Quintana and others have found that innate immune cells in the nervous system promote disease activity in MS. The team has identified a cell pathway where an intervention could potentially block the destructive properties of rogue innate immune cells and are testing potential agents for this purpose. Additionally, the network is examining a specific compound that could protect the brain from damage.

### Gaining New Insights and Developing Novel Approaches

Recognizing the opportunity to expand our work in understanding progression and accelerating treatment development, the Alliance is launching two new initiatives that will span into 2025:

- **Data Sharing Initiative** – leveraging the power of accumulated data from completed clinical trials could reveal new insights into progressive MS.

- **Innovative Experimental Clinical Trials** – current trials are too slow and too costly; this initiative will examine designing smarter trials and identifying mechanisms involved in progression so we can fail faster, learn and focus on winning areas.

### Integrating People with Progressive MS into the Drug Development Process

Incorporating the voice of people affected by multiple sclerosis into every stage of drug development increases the impact that treatments have on improving lives, and we must learn the best methods for doing so. This was among the important topics discussed at the January 2019 annual meeting of the International Progressive MS Alliance Industry Forum in Washington, D.C.

The Industry Forum is a partner and advisory group to the Scientific Steering Committee of the Alliance that contributes knowledge and expertise, and is composed of pharmaceutical industry representatives who have knowledge and expertise in the development of new therapies.

Outcomes from this meeting included:

- Expanding engagement of people affected by MS in Alliance initiatives, including the: Data Sharing Platform, Experimental Medicine Trial, Fluid Biomarker, Imaging and Functional Measures, and Rehabilitation and Symptom Management
- Continue to advocate for the engagement of people affected by progressive MS in the drug development process within all research settings

# ACCELERATING CLINICAL TRIALS

## MERGING SCIENCE AND CUTTING-EDGE TECHNOLOGY

### MRI Network

#### Predicting Progression Using MRI



Lead investigator **Professor Doug Arnold** from Canada in collaboration with investigators from Canada, The Netherlands, Switzerland, United Kingdom and United States, are working to develop imaging tools and computer programs that will

predict changes in the brain as MS progresses and provide understanding of how progression effects the brain. The tools could be used to evaluate whether a potential treatment is effective—is the treatment slowing or reversing damage in the brain—thus addressing a major barrier in current treatment development.

The team has assembled data from over 9,000 people that include more than 52,000 MRI scans. This pioneering work is being leveraged beyond the Alliances with significant collaborations being initiated in other research projects at the U.K. and U.S. MS societies.

### Speeding Clinical Trials

Current clinical trials take too long—often five years or more. Cutting down the amount of time will require new tools for quickly determining if a treatment is working. New initiatives are underway that supplement the MRI Network.

- **Fluid Biomarkers** – it is possible that a blood test could determine if a treatment is preventing nerve damage. Bringing experts together from necessary disciplines, we will accelerate work supporting fluid biomarkers and how they could be used in trials and the clinic.
- **Imaging and Functional Measures** – we will work to develop additional imaging techniques and other tests that can quickly assess a treatment’s effectiveness and perhaps, a patient’s prognosis.

“ This network is harnessing the enormous potential of machine learning and artificial intelligence to develop next generation MRI markers of disease progression that will be used in clinical trials. This work can help transform future treatment development.”

– **Professor Anthony Feinstein**

Canada, Scientific Steering Committee

## ENHANCING WELL-BEING

### MAKING A DIFFERENCE THROUGH REHABILITATION AND SYMPTOM MANAGEMENT

It’s rare to have people living with MS, family members, caregivers, physical and occupational therapists, brain researchers, psychiatrists and neurologists all in one room, sharing their points of view. That’s what happened when more than 225 researchers and supporters from 16 countries gathered in Toronto, Canada in May 2018 for the International Progressive MS Alliance Third Scientific Congress, “Making a Difference Through Rehabilitation & Symptom Management.” The result was a new perspective and resolve on urgent next steps to find solutions that will improve quality of life for people living with progressive MS.

Experts provided updates on approaches to restoring function, enhancing brain rewiring and addressing MS symptoms, including learnings from other disorders. Participants also discussed ways research is improved when study designs incorporate the perspective of the person living with the disorder being investigated. Rehabilitation and symptom management research is beginning to be translated into practice, but there is more work to do to find the best ways of enhancing well-being for people with progressive MS.

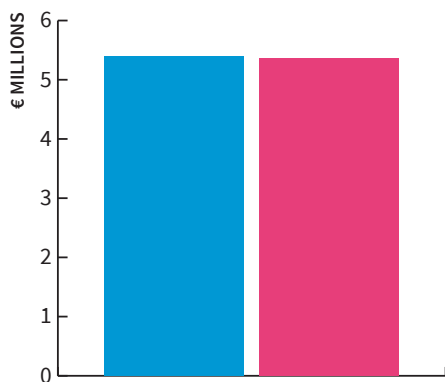
The Congress set the stage for the development of a global research strategy for well-being that will improve quality of life for people with progressive MS worldwide. The Alliance has formed an international team of experts that are focused on setting research priorities and building a roadmap through 2025.



# 2018 FISCAL YEAR FINANCIAL PERFORMANCE

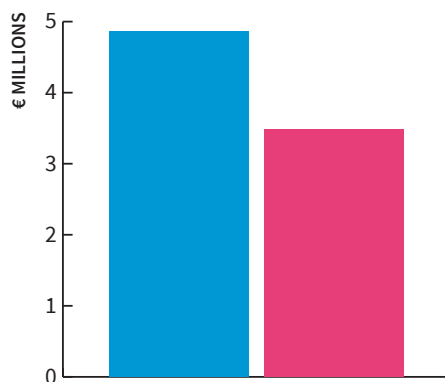
The Alliance continues to demonstrate strong financial performance because of growing membership and financial contributions. The Alliance has had a demonstrable impact on progressive MS in thought leadership, convening activities, collaborations and funding.

## REVENUE



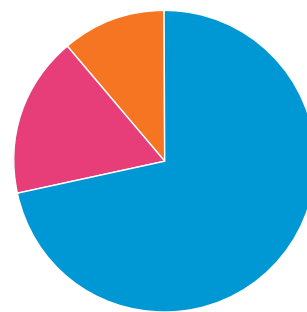
■ Budgeted: €5,399,244  
■ Actual: €5,368,842

## EXPENSE



■ Budgeted: €4,858,180  
■ Actual: €3,480,551

## EXPENSE ALLOCATION



■ Research grants: €2,487,459 (71%)  
■ Scientific strategic oversight and meetings: €597,812 (17%)  
■ Operations: €395,280 (11%)

**Total expenses: €3,480,551**



“ We must find a cure for progressive MS, and we believe the **best investment we can make is in the Progressive MS Alliance.** If we all join in, we can change the lives of millions.”

**– Cathy and Bill Onufrychuk**  
 Progressive MS Alliance Trust and Foundation Members

Photo, L to R: Bill and Cathy Onufrychuk with Professor Francisco Quintana, recipient of an Alliance Collaborative Network Award





Mirta, living with MS since 1997, Argentina



Bruce, living with MS since 2008, England



Manuella, living with MS since 2003, Germany



Barbara, living with MS since 2007, Italy

Together, we are stronger than progressive MS

# INTERNATIONAL PROGRESSIVE MS ALLIANCE

**CONNECT** TO END PROGRESSIVE MS

[info@ProgressiveMSAlliance.org](mailto:info@ProgressiveMSAlliance.org) [ProgressiveMSAlliance.org](http://ProgressiveMSAlliance.org)



Joseph, living with MS since 2008, U.S.



Kirsten, living with MS since 2001, Denmark



Brian, living with MS since 2005, Canada