



# Conference Report

2017 NMO ROUNDTABLE CONFERENCE  
& 2017 NMO PATIENT DAY





## 9th Annual International NMO Roundtable Conference

### The Power of Rare

The Guthy – Jackson Charitable Foundation’s 2017 International Roundtable Conference on NMO, “The Power of Rare”, was the foundation’s ninth and largest scientific event. Foundation-funded Principal Investigators, 80 International Clinical Consortium members, leading NMO clinicians and researchers from around the world, and members of industry were among those invited to the conference.

**The goals of the NMO Roundtable Conference were to:**

1. Learn about recent scientific and/or clinical advances in NMO research.
2. Reconvene the “best of the best” in neurodegenerative disease research for the purpose of continued information sharing and facilitating further strategic partnering for NMO research.
3. Align pharmaceutical, clinical and scientific strategies toward potential NMO drug discovery, and/or drug repurposing and/or clinical trials.

GJCF-funded Principal Investigators as well as other international scientists and clinicians presented their scientific findings and clinical progress to attendees with the intent of addressing the current and future status of scientific research, best clinical practices and pharmaceutical pathways. These presentations and panel discussions enhanced international collaboration to help advance the scientific and clinical knowledge of and work toward treatment and a potential cure for NMO.

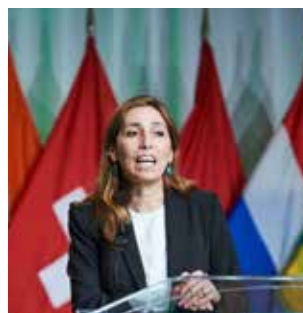
The NMO Roundtable Conference also featured meetings for the Biorepository Oversight Committee, CIRCLES, Definition and Severity of NMO Relapse (DSNR) Executive Committee, International Clinical Consortium, Industry Council, and a poster session featuring 33 submissions.



“

“Many thanks to you and your team for organizing a great meeting. Kindly convey my regards and a big thank you to Victoria Jackson and Bill Guthy for their enthusiasm and continued support to the cause.”

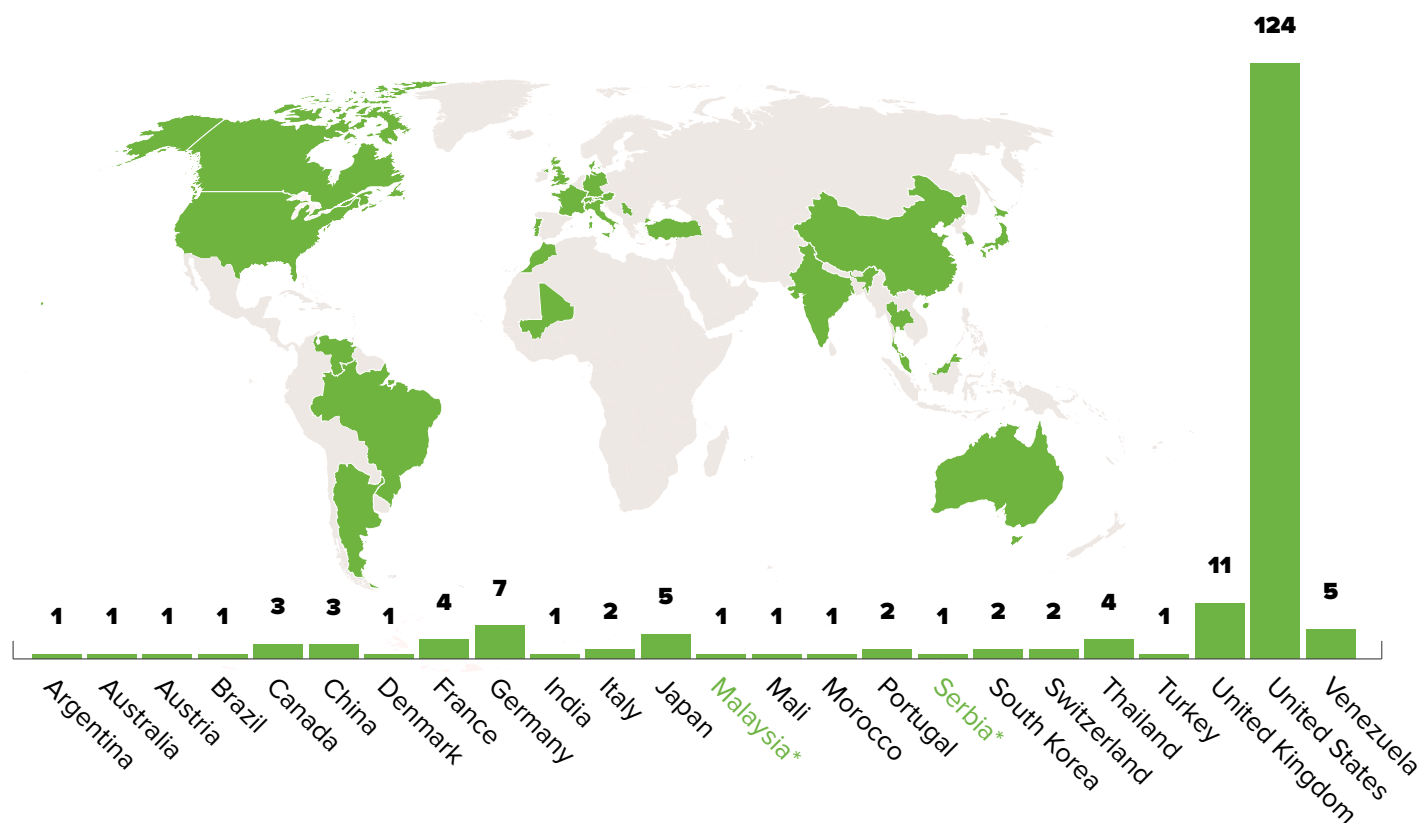
**- Maneesh Bhojak, MRCP, FRCR,  
The Walton Centre**







# Countries & Attendees

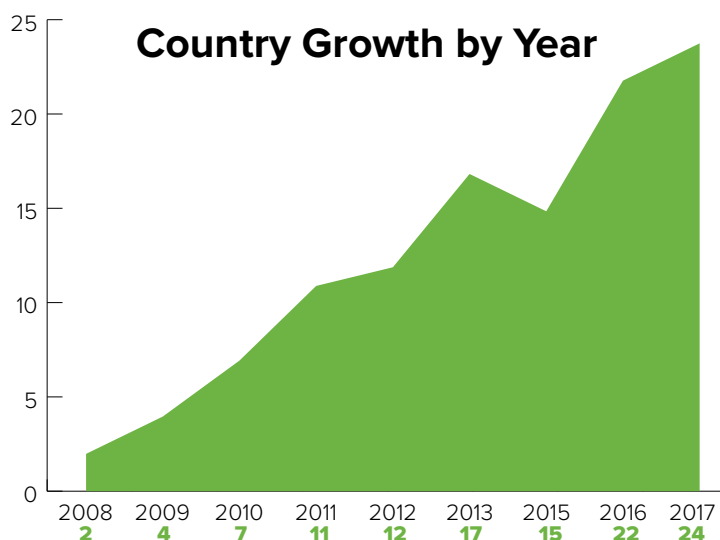


## 194 Attendees from 24 Countries

\*Green text indicates first time countries.

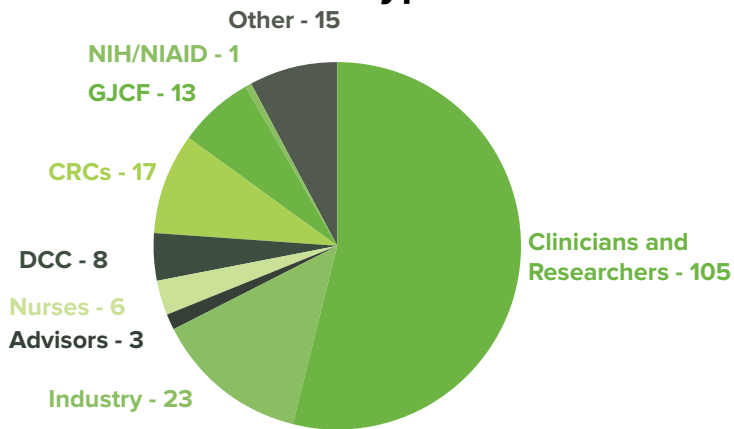
“What a wonderful conference that you folks setup! Thank you so much for including me and it was great to hear current research topics and get to meet the folks who are really moving the NMO field forward. The Guthy-Jackson Foundation is really pushing things forward in a powerful and meaningful way!”

- William Meador, MD,  
UAB Medicine

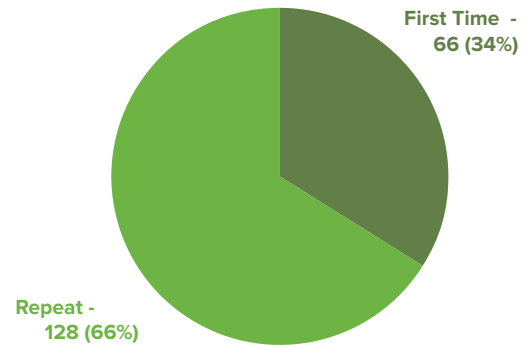


# Attendee Breakdown

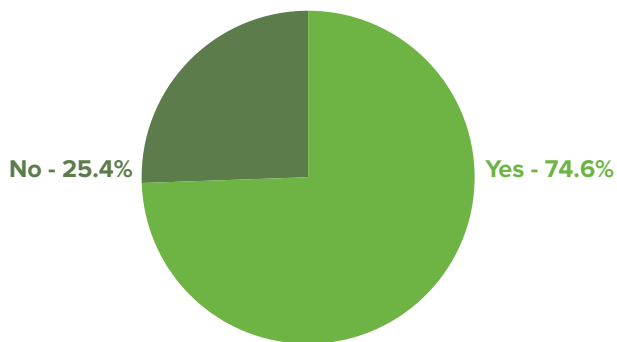
## Attendee Type



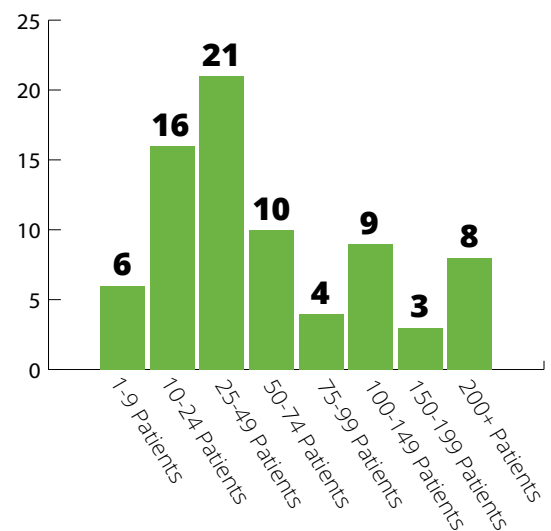
## First Time vs Repeat Attendees



Do you diagnose or treat NMO Patients?



If yes, approximately how many?



“

“I really enjoyed the scientific and humane ambiance at the conference. I think it’s a really meaningful event.”

- Che Serguera, MD, PhD, INSERM

# Organizations in Attendance

**Total Organizations 87**

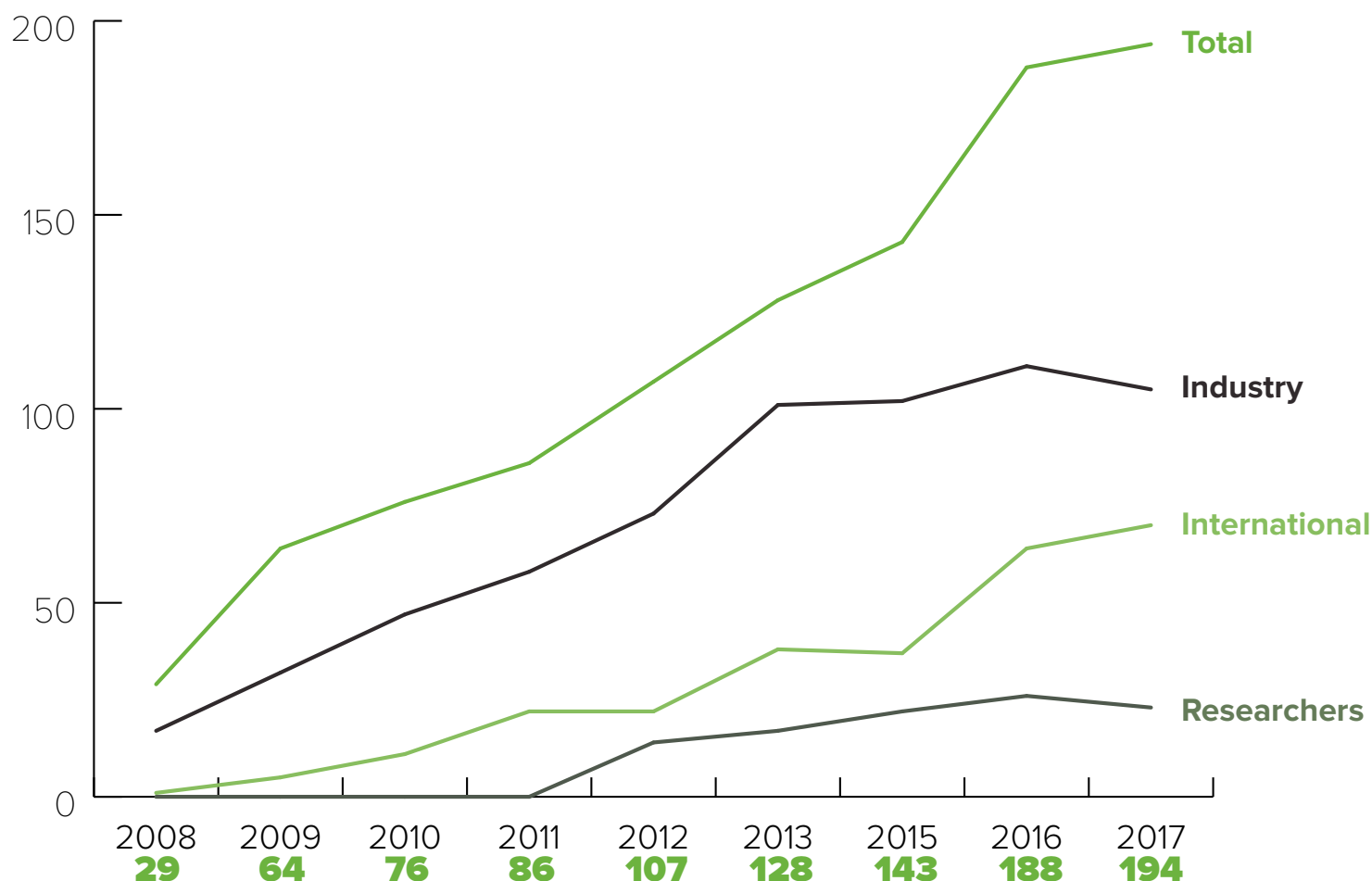
Alexion	Massachusetts General Hospital	University Hospital Düsseldorf
Brigham and Women's Hospital	Mayo Clinic	University of Alabama Birmingham
Caladrius	Medical University of Vienna	University of Bari
Caltech	MedImmune	University of Belgrade
Catholic University	Mount Sinai	University of British Columbia
Cedars-Sinai Medical Center	National Cancer Center	University of California, Irvine
Centro Hospitalar São João	National Center of Neurology and Psychiatry	University of California, Los Angeles
Centro Medico La Trinidad	National Pediatric Hospital J. P. Garrahan	University of California, San Francisco
Charite University	Niigata University	University of Colorado Denver
CHU Lyon	Nitte University	University of Istanbul
Chugai Pharmaceutical	NYU Medical Center	University of Miami
Cleveland Clinic	Oceana Associates	University of Michigan
Columbia University	Orion Bioscience	University of Missouri
Duke University	Oxford	University of Rochester
Genentech Roche	Penang General Hospital	University of Southern California
Google Verily	PPD	University of Southern Denmark
Great Ormond Street Hospital	Prasat Neurological institute	University of Technical Sciences and Technologies
Griffith University	Seoul National University Hospital	University of Utah
Harbor UCLA	Siriraj Hospital	University of Texas, Austin
Harvard University	St. Josef Hospital University of Bochum	UT Southwestern Medical Center
Hôpitaux de Paris	St. Michael's Hospital	VECTRIMS
Huashan Hospital	Stanford University	Yale
Immune Tolerance Network	Swedish Medical Center	
INSERM	TerumoBCT	
Ipsos	TG Therapeutics	
J. Craig Venter Institute / Human Longevity, Inc.	The Walton Centre	
John Radcliffe Hospital	Tohoku University	
Johns Hopkins University	Translational Medicine Partners, Inc.	
La Jolla Institute For Allergy and Immunology	Universidad Central de Venezuela	
Mahidol University	Universidade Federal de São Paulo	
Marrakech Medical School	Universität Düsseldorf	
	University Hospital Basel	

“I want to express my sincere gratitude to the entire GJCF team for another great and inspiring RT! As said in previous years, it is definitely the best conference of the year.”

**- Friedemann Paul, MD,  
Charité University**

# Eight-Year Meeting Attendance

**569%** Total Attendee  
Growth Since 2008



“This was really an outstanding meeting. It is amazing to see industry, clinical and basic science experts working in such a collaborative forum. For me the most impressive aspect was the very strategic way in which research is being supported and that the work that needs to be done is being done.”

- Simon Broadley, MD, PhD, Griffith University

# Conference Highlights



## **Keynote Speaker, J. Craig Venter, Ph.D.**

J. Craig Venter, Ph.D., is regarded as one of the leading scientists of the 21st century for his numerous invaluable contributions to genomic research.



## **Featured Speaker, Maria Spiropulu, Ph.D.**

Maria Spiropulu is an experimental physicist at the California Institute of Technology. She is a member of the CMS collaboration at the Large Hadron Collider where the Higgs boson was discovered in 2012.



## **Conference ePosters**

The foundation introduced a new digital platform for poster viewing this year. There were 33 ePosters submitted and available to view throughout the conference. Additionally, 9 posters were selected to be featured in a new “Rising Stars in NMO” session.



## **Audience Response System**

New this year, the foundation introduced sessions that featured a real-time electronic ballot system. Attendees were polled on provocative statements prior to and following invited speaker presentations. A goal of this unique approach was to identify specific hypotheses which if addressed would accelerate steps to curing NMOSD.





## 9<sup>th</sup> Annual International Roundtable Conference on NMO

Hilton Hotel LAX, Los Angeles / International Ballroom AB

March 13-14, 2017

### Conference Day 1

	7:00	Registration & Breakfast
Welcome	8:00	<i>NMO &amp; The Power of Rare – Ms. Victoria Jackson, Founder</i> <i>Six Dimensions of Rare – Michael Yeaman</i>
Keynote	8:30	<i>Human Genomics and the Future of Medicine – J. Craig Venter</i>
Session I		<u>Revolution: A New Era for NMOSD</u>
	9:00	Pathogenesis – <i>Jeff Bennett</i>
	9:25	Diagnosis – <i>Dean Wingerchuk</i>
	9:50	Epidemiology – <i>Lilyana Amezcua</i>
	10:15	Treatment – <i>Jackie Palace</i>
	10:40	Quality of Life – <i>Anu Jacob</i>
	11:05	Break
Session II		<u>Rethinking Rare to Solve NMOSD</u>
	11:20	Pioneering Omics to Decipher NMOSD – <i>Michael Yeaman</i>
	11:35	Genomic Clues in NMOSD Etiology – <i>Ben Greenberg</i>
	11:55	NMOSD Biomarkers: Challenge & Opportunity – <i>Dominic Spinella</i>
	12:20	Signatures of B cell Checkpoint Dysfunction in NMOSD – <i>Kevin O'Connor</i>
	12:40	Lunch
Session II (cont'd)		<u>Rethinking Rare to Solve NMOSD</u>
	1:30	NMOSD Through a Lens of Other Autoimmune Diseases – <i>Matthias von Herrath</i>
	1:55	Autoantibodies in Pediatric NMOSD – <i>Yael Hacohen</i>
	2:15	Restoring Immune Tolerance in NMOSD – <i>Jerry Nepom</i>
	2:40	Tolerization in Human NMOSD: One Step Closer – <i>Ché Serguera</i>
	3:00	Break





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#### Session III Illusion or Innovation in NMOSD ? \* *Real-Time Audience Interaction*

**3:15** **Decoding Etiology**

**Catalytic Concept:**

*NMOSD arises from a single heritable factor plus multiple acquired factors.*

Audience Vote / Lightning Round Speaker: **Vanda Lennon** / Forum / Audience Vote

**3:35** **Specifying Diagnosis**

**Catalytic Concept:**

*AQP4-reactive and MOG-reactive NMOSD are mutually exclusive diseases.*

Audience Vote / Lightning Round Speaker: **Kazuo Fujihara** / Forum / Audience Vote

**3:55** **Optimizing Efficacy**

**Catalytic Concept:**

*Treatment failures in NMO are due to specific immunologic mechanisms.*

Audience Vote / Lightning Round Speaker: **Takashi Yamamura** / Forum / Audience Vote

**4:15** **Tolerizing for Cures**

**Catalytic Concept:**

*Tolerization therapies will achieve cures for NMOSD within the next decade.*

Audience Vote / Lightning Round Speaker: **Larry Steinman** / Forum / Audience Vote

**4:35** **Enhancing Patient QoL**

**Catalytic Concept:**

*Inadequate emotional and quality of life care negatively impact NMOSD.*

Audience Vote / Lightning Round Speaker: **Nancy Sicotte** / Forum / Audience Vote

**Summary** **5:00** **Consilience – Michael Yeaman**

**Day 1 Adjourn – Ms. Victoria Jackson, Founder**

**5:30** Dinner

### Current GJCF Industry Council Members



### Emeritus GJCF Industry Council Members



## 9<sup>th</sup> Annual International Roundtable Conference on NMO

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March 13-14, 2017

### Conference Day 2

- 7:00** Breakfast & Headshots
- 8:00** Group Photo
- Opening 8:30** From Molecules to Miracles – *Ms. Victoria Jackson, Founder*  
Solving the Unsolvables – *Michael Yeaman*
- Feature 9:00** The Power of Rare Discoveries – *Maria Spiropulu*
- Session IV** Real-Time: Teamwork to Solve NMOSD \* *Real-Time Audience Interaction*
- Etiology & Epidemiology**  
**Catalytic Concepts:**
- 9:30** *A targetable mechanism is common to NMOSD & all autoimmune diseases.*  
Audience Vote / Lightning Round Speaker: *Simon Broadley* / Forum / Audience Vote
- 9:45** *New diagnostic & relapse criteria will transform NMOSD patient outcomes.*  
Audience Vote / Lightning Round Speaker: *Brian Weinshenker* / Forum / Audience Vote
- Autoantigens & Pathogenesis**  
**Catalytic Concepts:**
- 10:00** *NMOSD will be defined by a specific microbial & host antigen signature.*  
Audience Vote / Lightning Round Speaker: *Thomas Tedder* / Forum / Audience Vote
- 10:15** *The upstream cause of NMOSD is a dysfunction in central T cell tolerance.*  
Audience Vote / Lightning Round Speaker: *Scott Zamvil* / Forum / Audience Vote
- 10:30** Break
- Personalized Medicine**  
**Catalytic Concepts:**
- 10:45** *Pharmacogenomics are required to optimize therapeutic efficacy in NMOSD.*  
Audience Vote / Lightning Round Speaker: *Ho-Jin Kim* / Forum / Audience Vote
- 11:00** *Machine-learning image pattern recognition will transform NMOSD care.*  
Audience Vote / Lightning Round Speaker: *Alexander Brandt* / Forum / Audience Vote
- Drug Targets & Tolerization**  
**Catalytic Concepts:**
- 11:15** *Targeting upstream inflammation can reverse demyelination in NMOSD.*  
Audience Vote / Lightning Round Speaker: *Alan Verkman* / Forum / Audience Vote
- 11:30** *Autologous T reg therapy is a compelling strategy for treating NMOSD.*  
Audience Vote / Lightning Round Speaker: *Douglas Losordo* / Forum / Audience Vote
- 11:45** **Open Forum**



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### Conference Day 2

#### Session V Sharing the Power of Rare

##### 1:00 NMOSD Patient Experience Survey

Multi-Stakeholder Collaboration – *Jacinta Behne*

Preliminary Results – *Aysha Keisler*

##### 1:30 Patient Voices: Hopes & Concerns

Giving Voice to the Power of Rare – *NMO Patient Advocates*

Patient Poll Results – *Ali Guthy*

##### 2:00 Industry Council: Toward Approved NMOSD Therapies

2:00 Alexion – *Andrew Denker*

2:10 Chugai – *Athos Gianella-Borradori*

2:20 MedImmune – *Armando Flor*

##### 2:30 Rising Stars in NMOSD Research

Moderator – *Terry Blaschke*

5-Minute Lightning Round Poster Presentations

#### Conclusion

##### 3:30 LEAD: Campaign for a Cure – *Michael Yeaman*

##### 3:45 Power of Rare Movement – *Ms. Victoria Jackson, Founder*

##### 4:00 Mixer



## NMO Patient Day

March 15, 2017

8 am - 5 pm

Hilton LAX - International Ballroom AB





# Photo Gallery







# NMO Patient Day 2017

## The Power of Rare

The Guthy-Jackson Charitable Foundation's 8th Annual NMO Patient Day was held on March 15, in Los Angeles, CA, with the theme, "The Power of Rare." Founders Victoria Jackson and Ali Guthy opened the conference sharing their perspectives on what living with a rare disease was like nine years ago, and what it means to them now, nearly 10 years later.

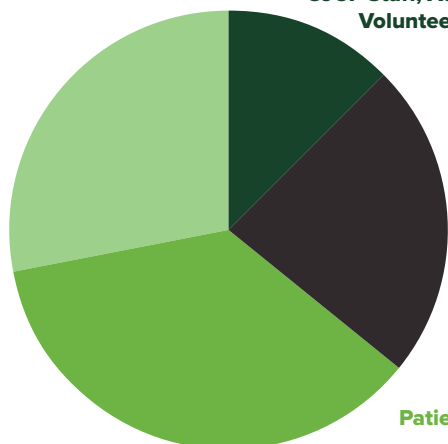
### Total Attendees - 259

Caregivers, Family,  
& Friends - 72 (28%)

GJCF Staff, Advisors, Founders,  
Volunteers & VIPs - 33 (13%)

Clinicians, Researchers,  
Industry - 60 (23%)

Patients - 94 (36%)



"While NMO is a rare disease," Co-Founder Victoria Jackson said addressing the audience, "in those early years feeling so powerless, nine years later we've been able to find the power in being rare." It was through research over those years she discovered, "what we think is so rare, isn't necessarily so rare."

For Ali Guthy, "The Power Of Rare" means, "building this tremendous community, and the strength we found in each other – working towards building a promising future for NMO."

The conference included a CIRCLES NMO repository blood draw, information about NMO clinical trials, the hallmark "Ask the Docs" session, preliminary results from the foundation's quality of life survey, breakout sessions and sessions led by NMO-Advocate attendees.

Videos of NMO Patient Day are available for free public access on the foundation's website and YouTube channel.



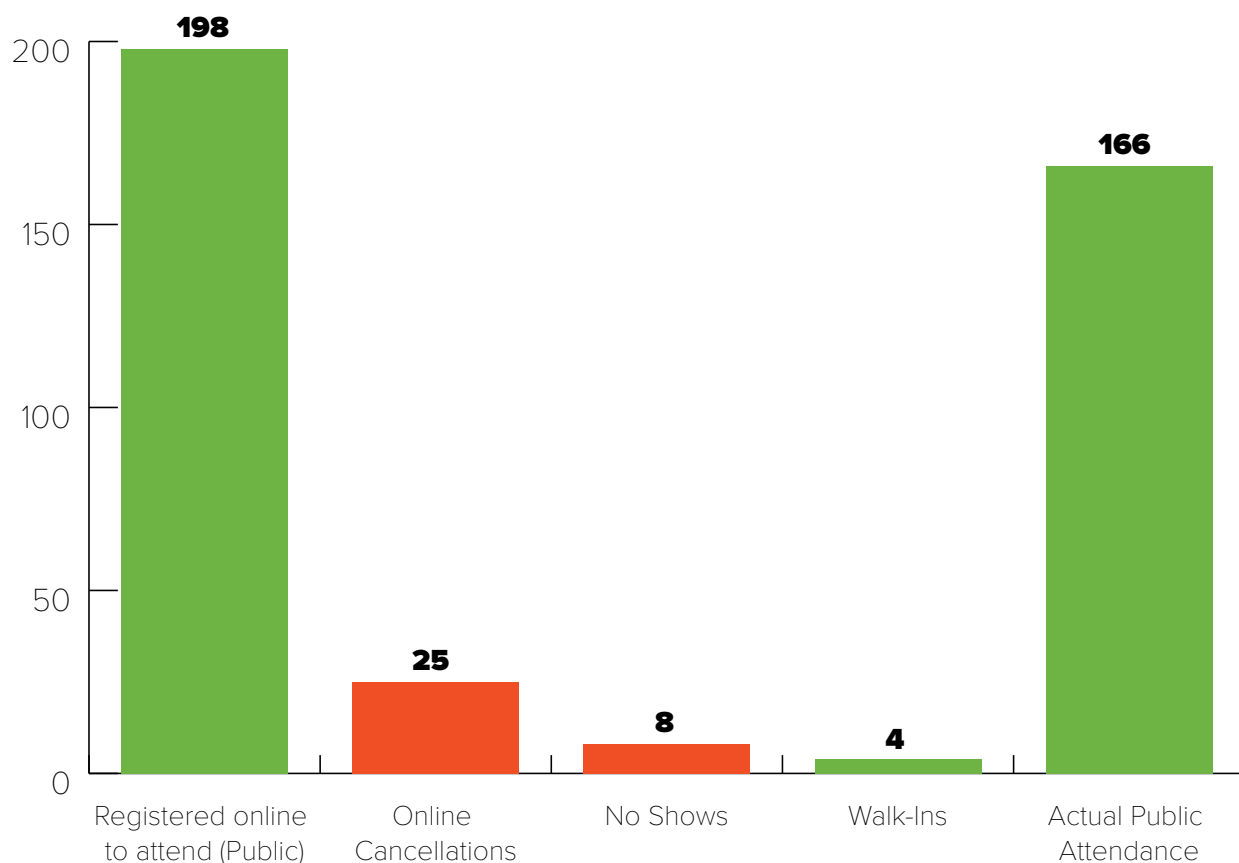
“Thank you so very much for having me there. I met some super people and had an amazing time. I made friends, learned about what to do in an attack and ‘being scared and alone’ was replaced with ‘I got this, I have control.’ I know NMO and am no longer afraid.”

**- Betty L. Friis-Halcomb**

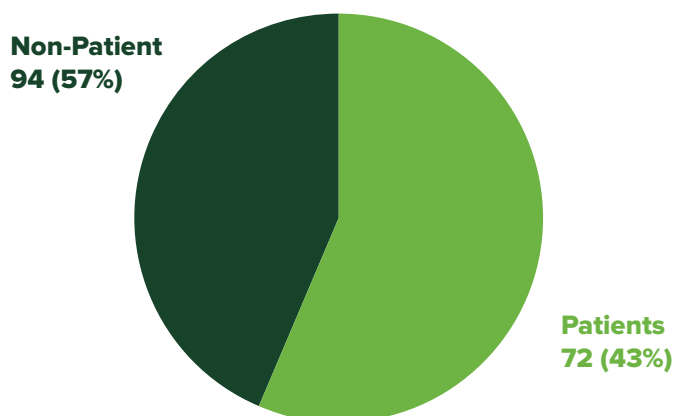




# Attendee Breakdown



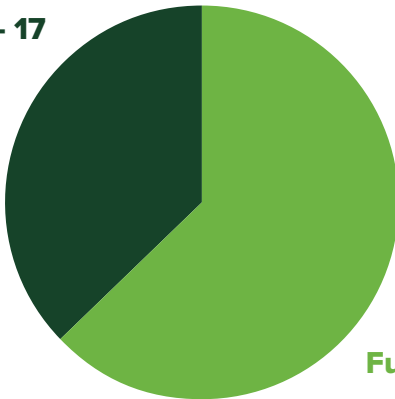
## Patient to Non-Patient Ratio



# Attendee Breakdown Cont.

## Travel Grants

Partial - 17

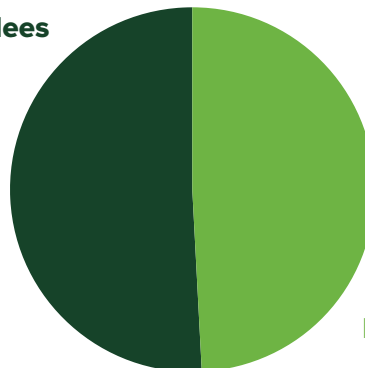


Full - 29



## First-Time vs Repeat Attendees

Repeat Attendees  
84 (51%)

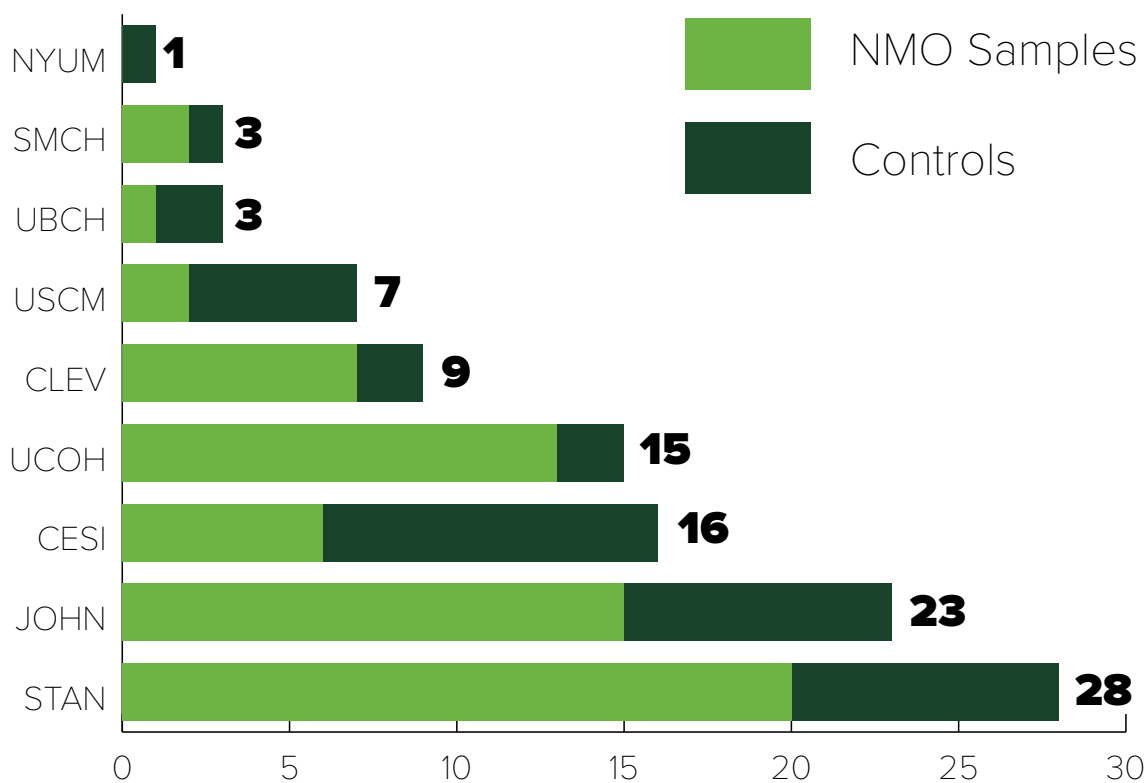


First-Time Attendees  
82 (49%)

# Blood Draw

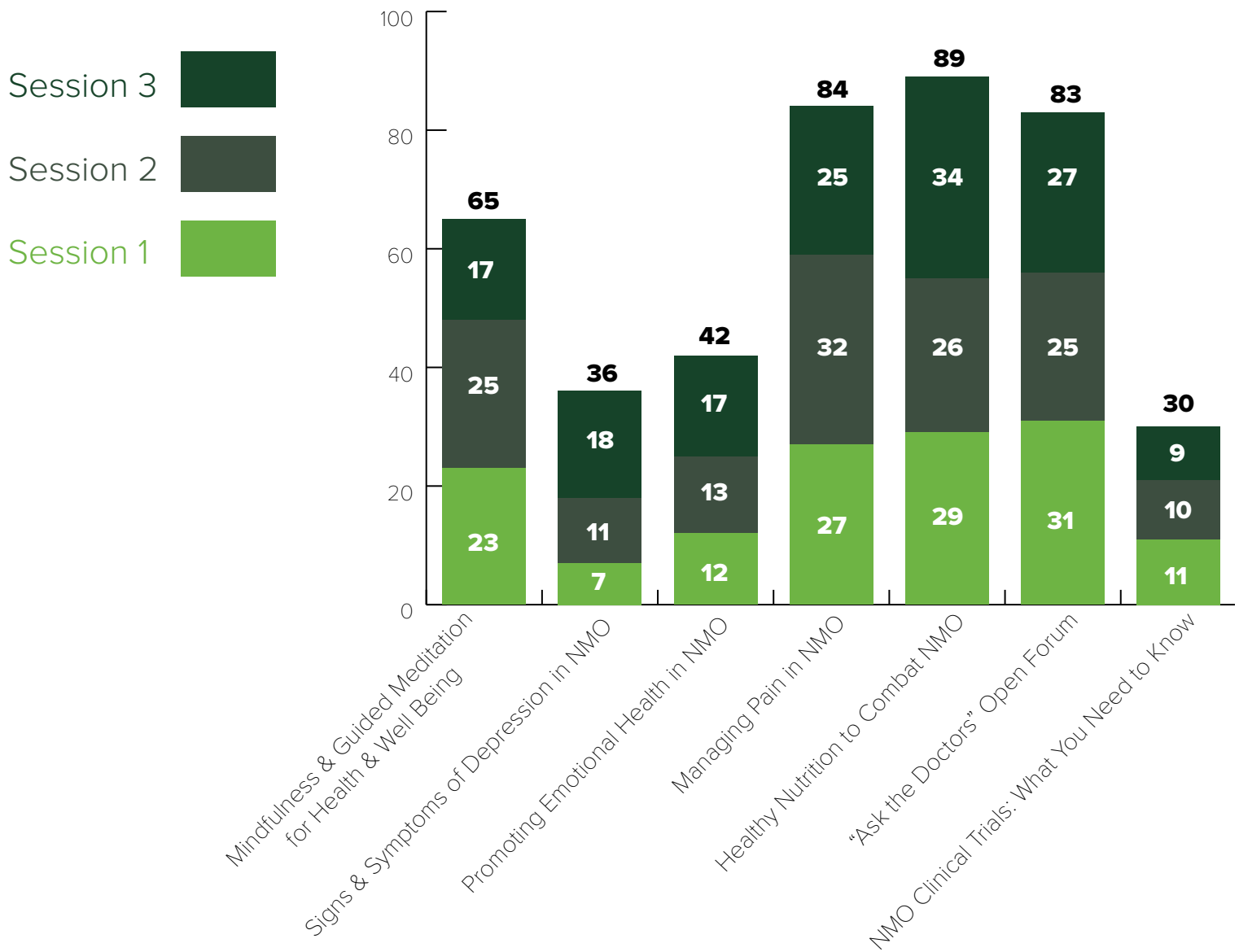


**105 Total Blood Draws** & **58 NMO Samples**





# Breakout Session Attendance



\*Indicates first two sessions only.



# Featured Resources



**Power of Rare Video Premiere**



**NMO Message Wall and Thumbprint Tree**

**Free Literature including *Saving Each Other*, *The Sleep Revolution*, and *Wake Up to the Joy of You***



# Media

## WONDROS Interviews Onsite

10 NMO patient stories captured



## Social Media

20K FB reach for 9 posts over a 5-day-period







## 8<sup>th</sup> Annual NMO Patient Day

MARCH 15, 2017 LAX HILTON HOTEL | INTERNATIONAL BALLROOM AB

### GJCF NMO CIRCLES Biorepository Blood Draw

MARCH 14 & 15 - PACIFIC BALLROOM A

- 7:00 a.m. **Registration** International Ballroom A Lobby  
**Breakfast** International Ballroom AB
- 8:00 **NMO Patient Day Welcome**  
*The Power of Rare* – Ali Guthy & Victoria Jackson, Founders
- 8:15 **You are the Cure** – Dr. Michael Yeaman
- 8:30 **Q&A: Ask the Doctors**
- Session 1** Drs. Dorlan Kimbrough, Maria Leite, Michael Levy, Anne-Katrin  
**Panelists** Pröbstel, Nancy Sicotte, Larry Steinman, Dean Wingerchuk
- Session 2** Drs. Jeffrey Bennett, Robert Carruthers, Yang Mao-Draayer, Pavle  
**Panelists** Repovic, Silvia Tenenbaum, Brian Weinshenker, Scott Zamvil
- 10:00 **Break**
- 10:20 **Q&A with Ali Guthy & Victoria Jackson**
- 10:35 **A Unique Collaboration: NMO Patient Experience Survey**  
**Model of Teamwork** – Jacinta Behne  
**Preliminary Results** – Aysha Keisler, Ipsos
- 11:00 **Clinical Trials – Progress Toward Approved NMO Therapy**  
**Program Updates / Audience Q&A: Alexion, Chugai, MedImmune**  
Drs. Andrew Denker, Athos Gianella-Borradori, Armando Flor
- 11:45 **CIRCLES: Patients Enabling Breakthroughs**  
**Program Expansion** – Jacinta Behne  
**Your Biobank at Work** – Dr. Michael Yeaman
- 12:00 p.m. **Buffet Lunch** International Ballroom AB



**1:30 p.m. Breakout Sessions: The Power of Rare**

- 1. Mindfulness & Guided Meditation for Health & Well Being – Marina Room**  
**Presenter** Agapi Stassinopoulos, Author & Speaker
- 2. Signs & Symptoms of Depression in NMO – Los Angeles A**  
**Presenters** Drs. Alexander Brandt, Nancy Sicotte & NMO Advocates
- 3. Achieving Wellness in NMO – Los Angeles B**  
**Presenter** Dr. Ira Lesser
- 4. Managing Pain in NMO – Los Angeles C**  
**Presenters** RNs. Erin Fromm, Sandra Reeve & NMO Advocates
- 5. Healthy Nutrition to Combat NMO – Carmel Room**  
**Presenters** Dr. Marco Vespignani
- 6. “Ask the Doctors” Open Forum – La Jolla Ballroom B**  
**Panelists** Drs. Robert Carruthers, Stacey Clardy, May Han, Esther Melamed, Pavle Repovic, Silvia Tenenbaum
- 7. NMO Clinical Trials: What You Need to Know – La Jolla Ballroom A**  
**Presenters** Drs. Jeffrey Bennett & Michael Yeaman

**3:20 Break International Ballroom AB**

**3:50 Giving Voice to the Power of Rare – Ali Guthy & Advocates**

**4:30 Saving & Improving Lives – Dr. Michael Yeaman**

**4:40 A Power of Rare Movement – Victoria Jackson, Founder**

**4:45 Group Photo & Adjourn**

# Photo Gallery

