

2018 REiNS Winter Meeting Survey Results

Designing Clinical Trials for Cutaneous
Neurofibromas, An Unmet Medical Need for Patients
with Neurofibromatosis 1



Response Evaluation In Neurofibromatosis Schwannomatosis
INTERNATIONAL COLLABORATION

Attendees

Stakeholders

- Rosemary Anderson
- Diana Haberkamp
- Pam Knight
- Karen Peluso
- Traceann Rose
- Lori Ryan
- Connie Sorman

Researchers

- Mimi Berman **
- Jaishri Blakeley*
- Ashley Cannon*
- Peter DeBlank
- Michael Fisher
- Kathy Gardner
- Andrea Gross
- Marlene Guiraud
- Uchenna Iloeje
- Jennifer Janusz
- James Lee
- Ina Ly
- Staci Martin
- Vanessa Merker
- Dominique Pichard*
- Scott Plotkin*
- Alyssa Reddy
- Jens Renstrup
- Melissa Reyes*
- Sameer Farouk Sait
- Kavita Yang Sarin*
- Raquel Thalheimer
- Heather Thompson
- Sharad Verma
- Deeann Wallis
- Karin Walsh
- Jess White
- Brigitte Widemann
- Pam Wolters
- Michael Wootton
- Kaleb Yohay
- Ann Blanton **

Patient Representatives

- Sarah Adsit
- Brandie Evans **
- Tammy Benson O'Brien
- Dale Berg
- Alexandra Cellucci
- Roberto da Silva Ribeiro **
- Gregg Erickson*
- Onno Faber
- Maureen Hussey
- Andres Lessing
- Beverly Oberlander
- Class Rohl
- Jessica Samblanet
- Herb Sarnoff

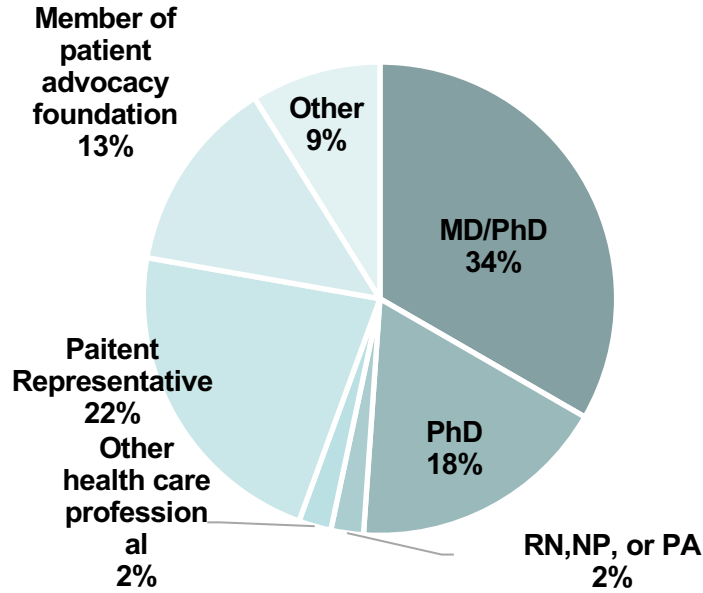


* Indicates speakers

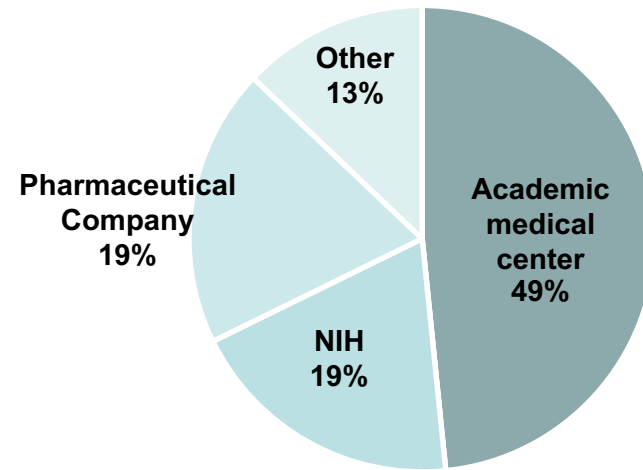
** Indicates attendees who joined via GoToMeeting

- Attendees: 49 in person, 4 by teleconference
- Respondent Demographics (n=45/53, 85%):

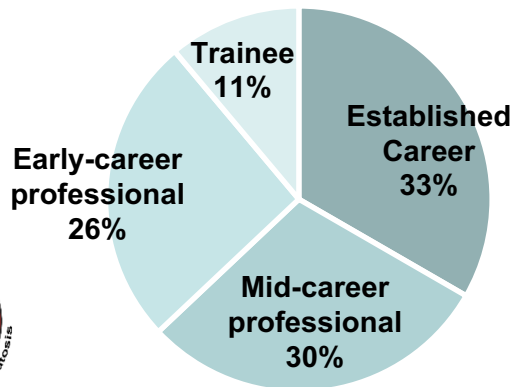
Title/Profession



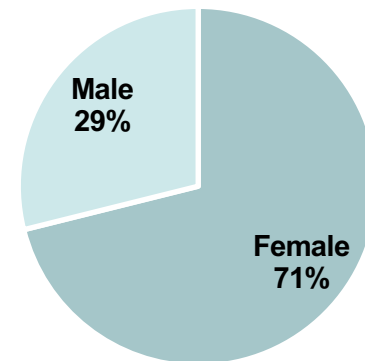
Institutional Affiliation



Career Stage



Gender Identity



Meeting Objectives

Percent of attendees that either “Agreed” or “Strongly agreed” that the program content achieved the following objectives:

“Disseminate working group recommendations for primary and secondary endpoints for clinical trials of cutaneous neurofibromas” :

- 96% of all attendees
 - 94% of researchers and clinicians (51% strongly agreed)
 - 100% of patient representatives (40% strongly agreed)

“Enhance understanding of eligibility criteria for trial designs with a focus on trials for small and large cutaneous neurofibromas”:

- 95% of all attendees
 - 91% of researchers and clinicians (38% strongly agreed)
 - 100% of patient representatives (70% strongly agreed)

“Integrate the views of key stakeholders (investigators, patient representatives, pharmaceutical companies, and members of the Food and Drug Administration) to define clinical benefit and relevant endpoints in cNF”:

- 98% of all attendees
 - 97% of researchers and clinicians (74% strongly agreed)
 - 100% of patient representatives (70% strongly agreed)



Percent of Information that was New to Attendees

	0-25%	26-50%	51-75%	76-100%
All Attendees (N=45)	18%	51%	22%	9%
Researchers (N=35)	17%	54%	20%	9%
Patient Representatives (N=10)	20%	40%	30%	10%



Activity Satisfaction

Percent of attendees that responded “Very Good” or “Excellent” to the following categories:

For overall organization of the conference

- 98% of attendees (71% excellent)
 - 97% of researchers and clinicians (74% excellent)
 - 100% of patient representatives (60% excellent)

For overall quality of the conference

- 100% of attendees (67% excellent)
 - 100% of researchers and clinicians (66% excellent)
 - 100% of patient representatives (70% excellent)



Activity Satisfaction Continued

Percent of attendees that responded “Strongly Agree” or “Agree” to the following statements:

The conference was an appropriate duration (1 day):

- 93% of attendees (58% Strongly Agree)
 - 94% of researchers and clinicians (57% Strongly Agree)
 - 90% of patient representatives (60% Strongly Agree)

Agreed the conference venue met their needs:

- 84% of attendees (51% Strongly Agree)
 - 83% of researchers and clinicians (51% Strongly Agree)
 - 90% of patient representatives (50% Strongly Agree)

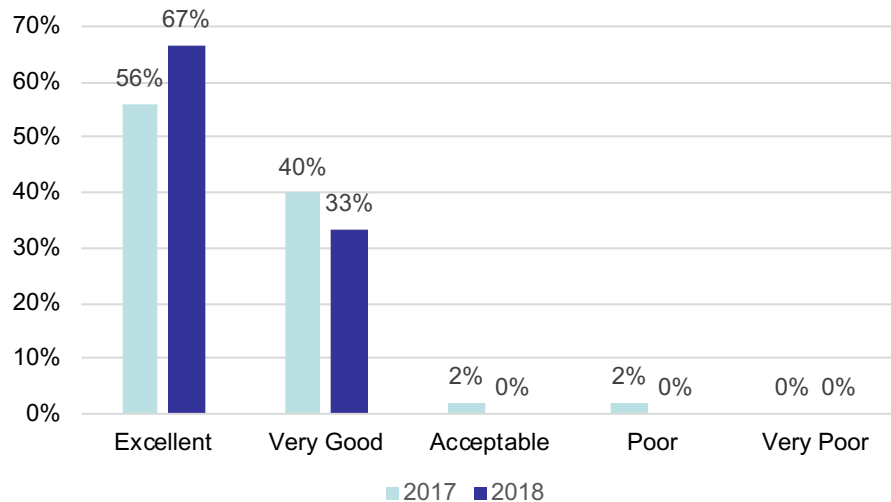


Activity Satisfaction: 2017 vs. 2018

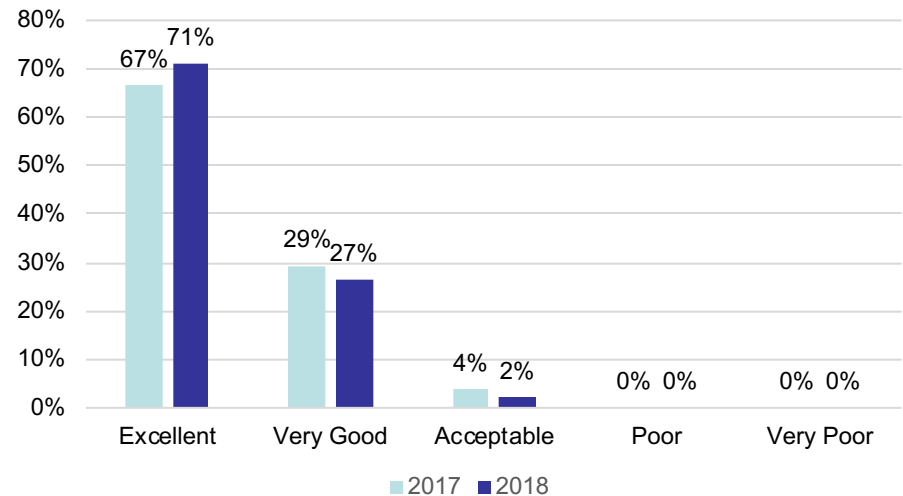
2017: N=53 survey respondents

2018: N=45 survey respondents

The **Overall Quality** of the Conference was:



The **Overall Organization** of the Conference was:

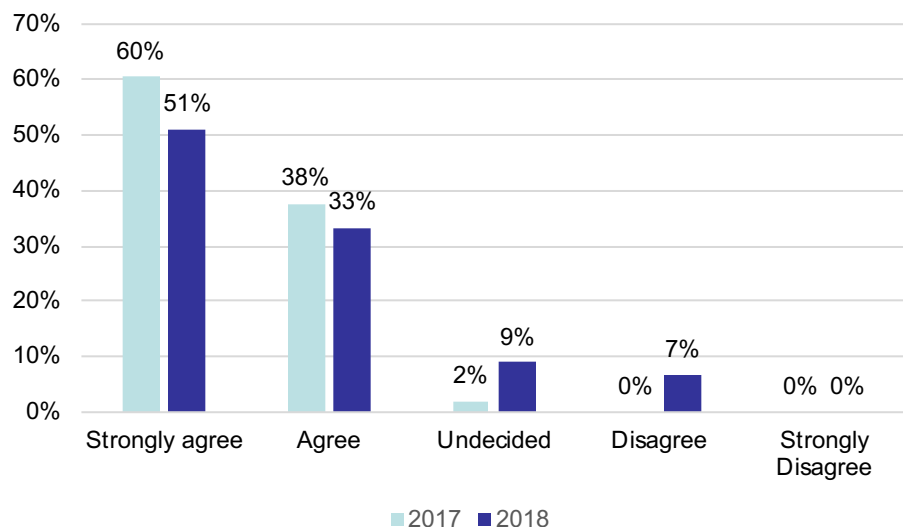


Activity Satisfaction: 2017 vs. 2018 continued

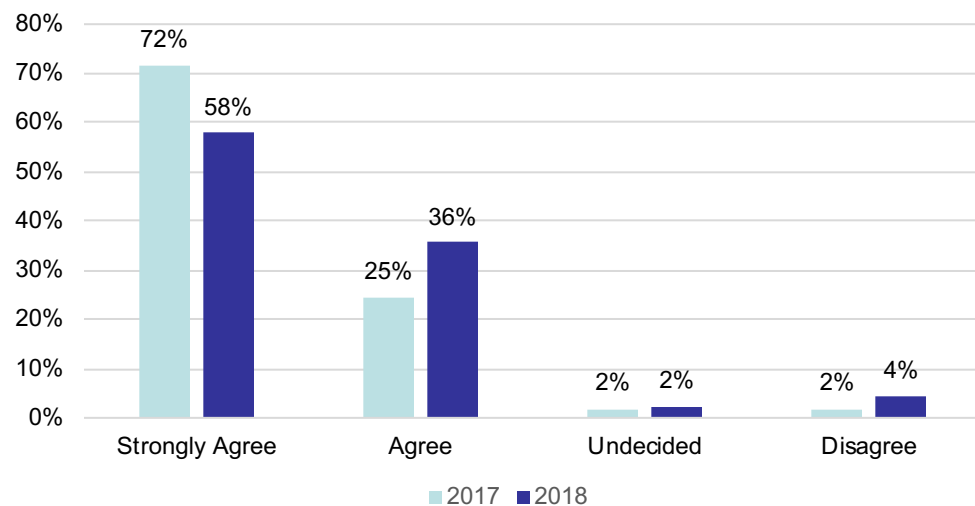
2017: N=53 survey respondents

2018: N=45 survey respondents

The Conference Venue Met My Needs:



The Conference was an Appropriate Duration (1 day):



Activity Satisfaction: Sunday Educational Dinner for Patient Reps

Percentage of attendees who “Strongly Agree” or “Agree” to the following statement:

The information presented during the Sunday dinner helped me better understand Monday’s meeting:

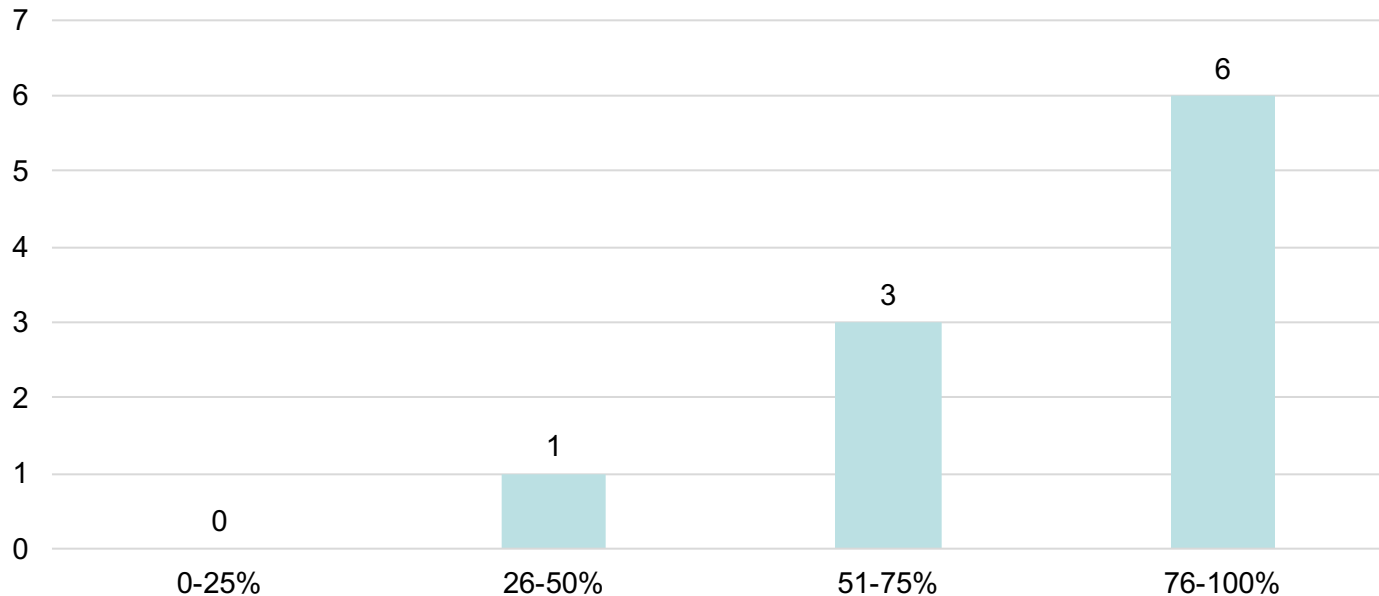
- 88% Agree (63% Strongly Agree)



Patient Representatives Only:

All patient representatives (N=10) reported being able to read some of the online patient representative educational materials.

What percentage of the written core educational materials were you able to read?



9/10 patient representatives were able to read 50% or greater of the educational materials.



Patient Representatives:

Percent of attendees that responded “Excellent” or “Very Good” to the following statements:

The overall quality of the written educational content:

- 100% of patient representatives (30% Excellent)

Educational material length:

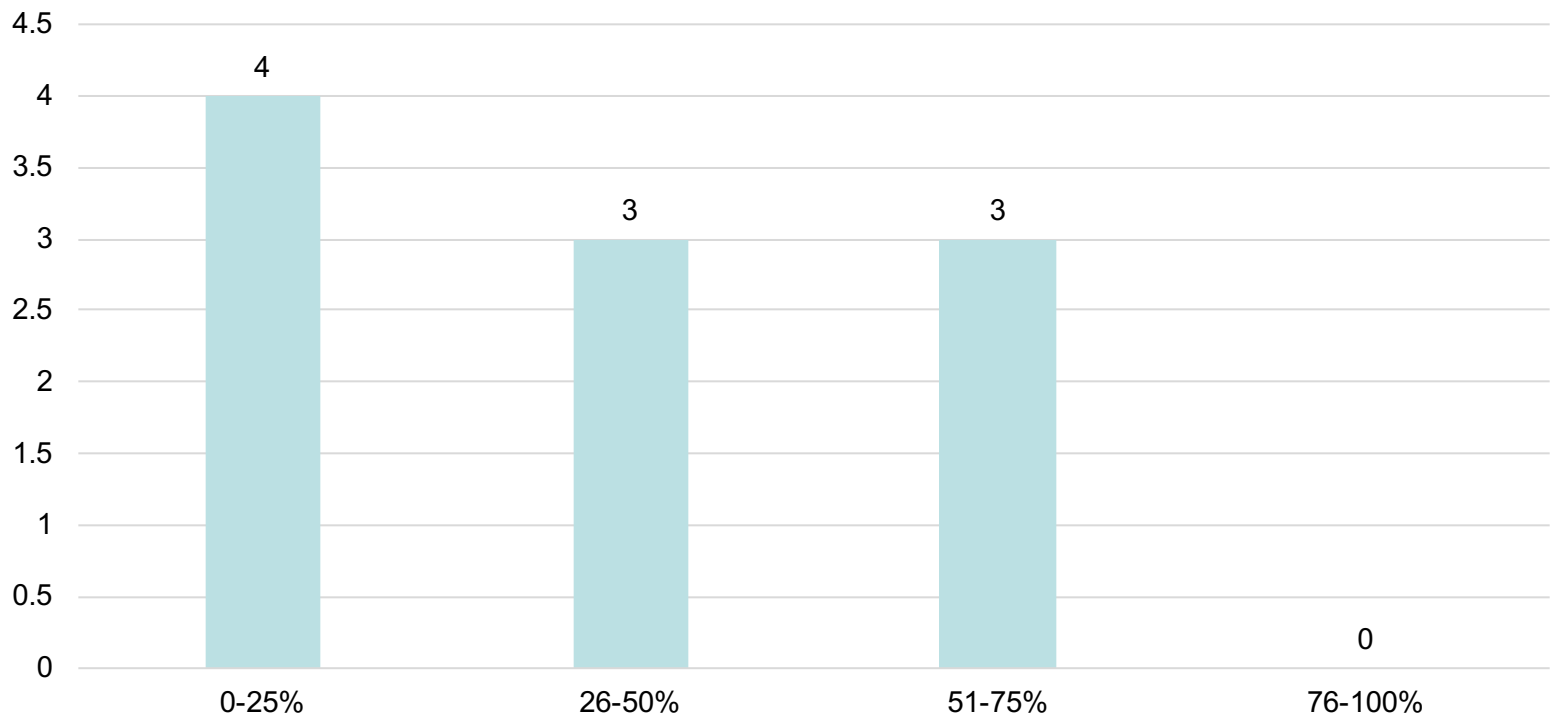
The written educational material was an appropriate length:

- 60% “Agree”
- 20% “Undecided”
- 20% “Disagree, the current version is too long”



Patient Rep Educational Materials

What percentage of the written educational materials was new to you?



Constructive Comments

- Venue
 - Venue is okay, but room/set up could have been improved
 - Hotel does not seem like a good value
 - Expensive
- More Patient Centered
 - Glossary of frequently used terms
 - Encourage researchers to explain terms clearly to non-experts (did a good job at start of the meeting but faded throughout the day)
- Topic and speaker suggestions
 - Consider having 1-2 additional guest speakers
 - Add updates from all of the subgroups and their activities and timelines
 - Patient representatives may benefit from reviewing or presenting basic biochemistry and how biomarkers are found and used
 - Add another topic and reduce overlap between presentations



Praise

- “This was a great conference, each year I think this was the best but it keeps getting better.”
- “Great job overall.”
- “I really appreciate this being available electronically so that I could participate.”
- “Thank you for having meetings like this one. I learned so much today”
- “This was a fabulous conference that brought together key stakeholders to make essential progress in providing a strong foundation to support clinical trials in this condition (cutaneous neurofibromas).”

