

Patient Representation Initiative for REiNS

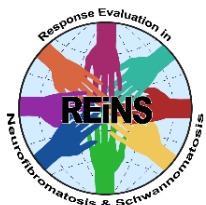
Initial results



Response Evaluation In Neurofibromatosis Schwannomatosis
INTERNATIONAL COLLABORATION

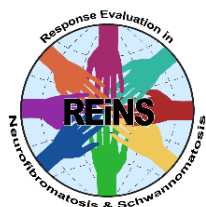
Process

- Online application developed by Pam Wolters, Scott Plotkin, Traceann Rose
- Online application available on 9/16/2017
- Application deadline 10/16/2017
- Includes personal statement and letter of recommendation

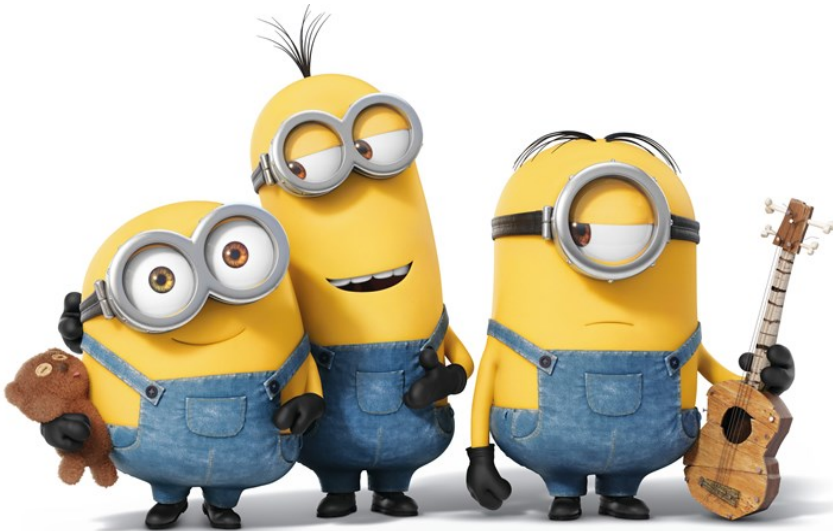


Response

- 54 online applications completed
- 30/54 (56%) applications completed including personal statement and letter of recommendation
- NF representation
 - NF1: 17/30 (57%)
 - NF2: 11/30 (37%)
 - SWN: 2/30 (7%)



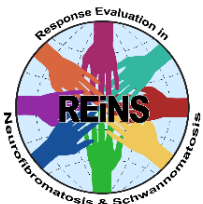
Patient representative demographics



Patient: 17/30 (57%)
Parent: 12/30 (40%)
Spouse: 1/30 (3%)

Race:
White: 28/30 (94%)
Asian: 1/30 (3%)
Mixed: 1/30 (3%)

Ethnicity:
Not hispanic: 27/30 (90%)
Hispanic: 3/30 (10%)

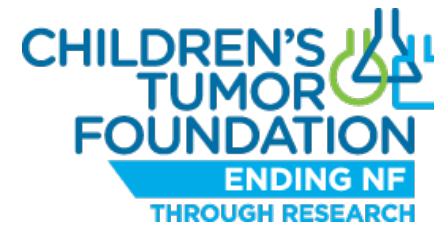
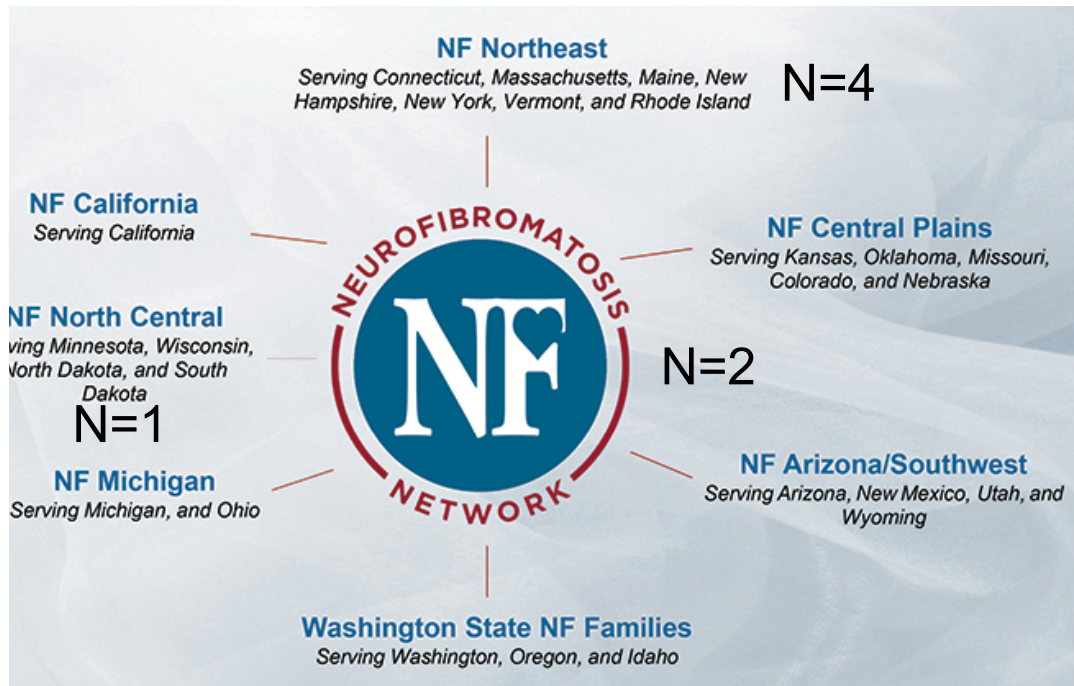


Highly educated cohort



Graduate school: 16/30 (53%)
College: 11/30 (37%)
High School: 3/10 (10%)

Employee or volunteer of organization



N=4



N=3

NF TEXAS
NEUROFIBROMATOSIS
FOUNDATION®



NF Kinder N=1

Neurofibromatose durch Forschung besiegen



N=1



N=1



New Futures Leaders: 1
No affiliation: 11

Results

- How did you hear about opportunity?
 - Email invitation: 13
 - NF Foundation: 9
 - CTF: 4
 - Health professional: 4



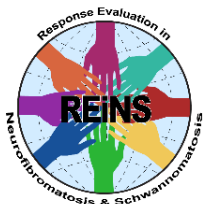
Applicant interests

Top choice

- 1) Patient reported outcomes (30%)
- 2) Disease biomarkers (20%)
- 3) Tumor imaging (13%)
- 4) Functional outcomes (10%)
- 5) Whole body MRI (7%)
 - Neurocognitive outcomes (7%)
 - Visual outcomes (7%)
- 8) Cutaneous neurofibromas (3%)

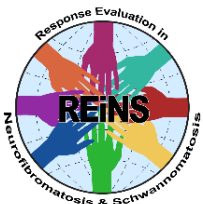
Top 3 choice

- 1) Patient reported outcomes (63%)
- 2) Disease biomarkers (40%)
 - Tumor imaging (40%)
- 4) Functional outcomes (37%)
- 5) Cutaneous neurofibroma (33%)
- 6) Whole body MRI (30%)
- 7) Neurocognitive outcomes (20%)
- 8) Visual outcomes (13%)



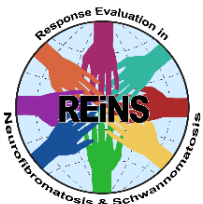
Working group allocations

- Based on patient representative preference
 - Patient reported outcomes: 3
 - Functional outcomes: 4
 - Tumor imaging: 3
 - Whole body MRI: 4
 - Visual outcomes: 2
 - Neurocognitive outcomes: 3
 - Disease biomarkers: 5
 - Cutaneous neurofibromas: 3



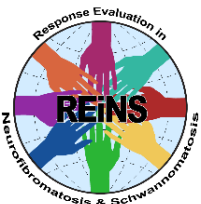
Training roadmap

- November
 - Distribute REiNS supplements for background
 - Teleconference(s)
 - Introduction to REiNS: mission and organization
 - Background on patient representation
 - Review goals for December meeting
- December
 - Learn from FDA training
 - Feedback from first meeting
 - PRs to help generate training focus
 - Webinars with Maarten de Wit, PhD about patient engagement



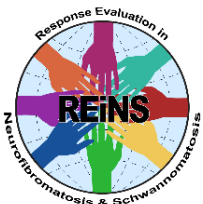
Training

- January-April
 - Establish patient representation working group with ALL PRs
 - Leadership: 2 PRs and some REiNS leaders
 - Goals:
 - Develop training
 - Define goals of PRs for REiNS
 - Develop financial model



Comments

- Anticipate that some members of current class will change groups or decide not to participate during first year
- Role/training of PR to develop over first year
- Need more diversity
- Paper to be developed for next REinS supplement



Acknowledgements

- Pam Wolters
- Vanessa Merker
- Traceann Rose
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