

Response Evaluation In Neurofibromatosis Schwannomatosis
INTERNATIONAL COLLABORATION

- If sharing any data or information from these slides generated by the REiNS International Collaboration, please acknowledge the authors, group chairs, and specific working group.
- If using any information presented with a citation, please reference the primary source.



Using patient views to inform the design of clinical trials for cNF

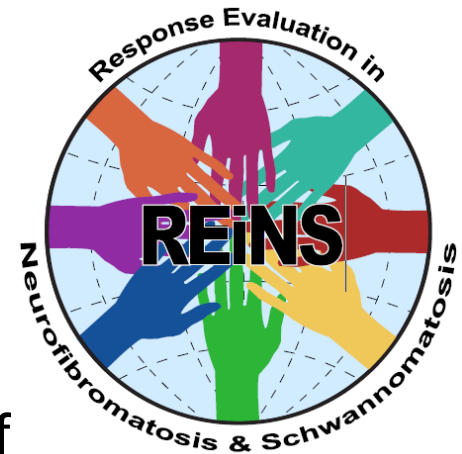
Cutaneous Neurofibroma Working Group
Ashley Cannon, PhD, MS, CGC



Response Evaluation In Neurofibromatosis Schwannomatosis
INTERNATIONAL COLLABORATION

Adult cNF survey

- Created by the REiNS Cutaneous Neurofibroma Working Group
 - Reviewed and piloted by REiNS patient representatives
 - FDA provided feedback (Melissa Reyes, MD)
- Designed to collect basic demographic information, details about the patient's cNFs, views on morbidity related to specific aspects of cNFs, and views regarding current and potential future cNF treatment
- A survey link was distributed via the Children's Tumor Foundation Patient Registry email blast
- Inclusion criteria: NF1, at least 1 cNF, at least 18 years old, and English speaking

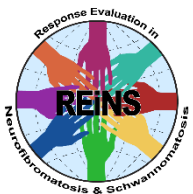


JOIN THE
NF REGISTRY

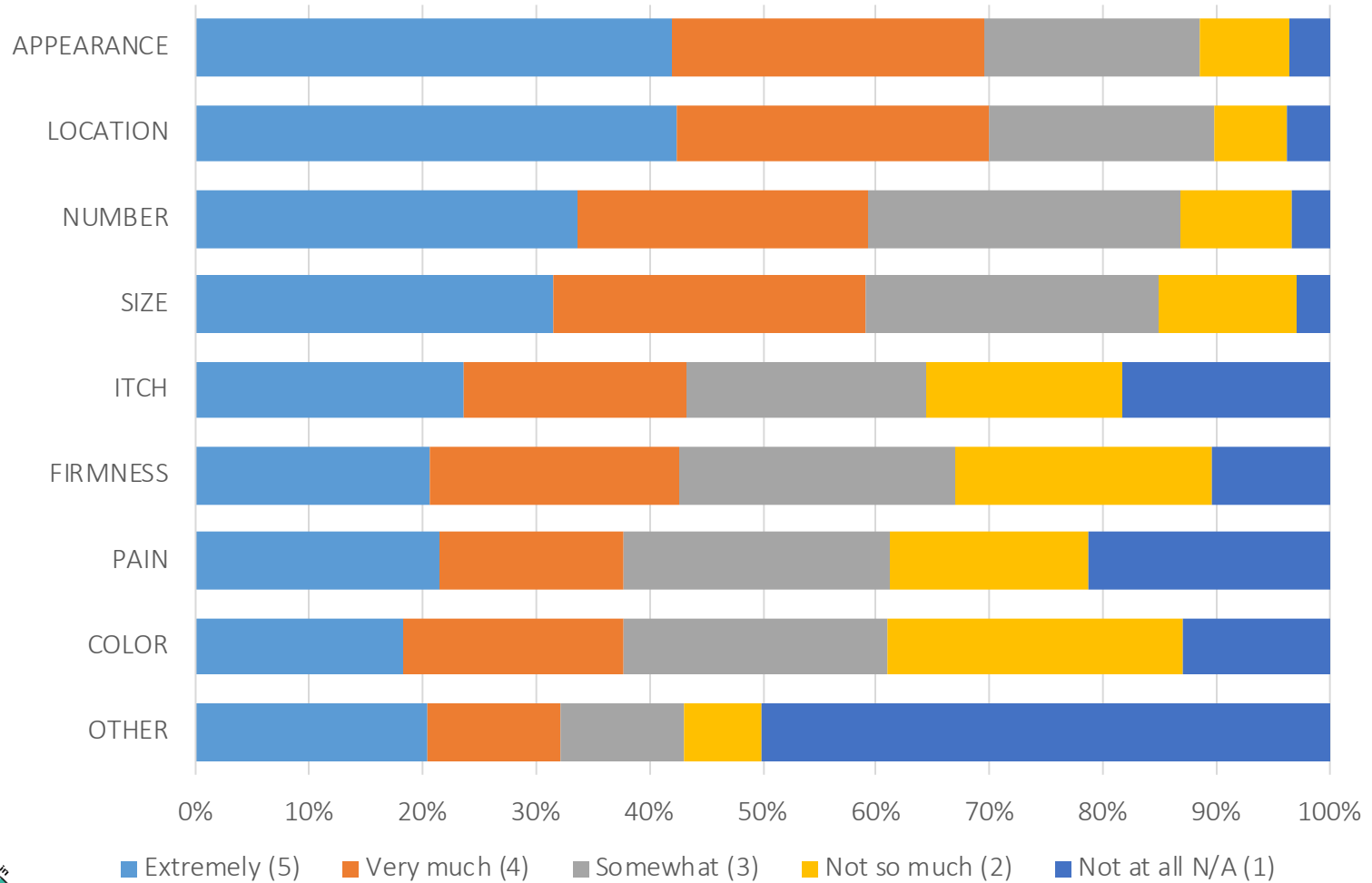
www.nfregistry.org



Characteristics (n=548)	Mean (SD) or N (%)
Age	44.9 (\pm 13.7)
Sex	
Female	385 (67.7%)
Male	171 (30.1%)
Other	1 (0.2%)
Not answered	12 (2.1%)
Race	
American Indian or Alaska Native	7 (1.2%)
Asian	20 (3.5%)
Black or African American	15 (2.6%)
Native Hawaiian or Other Pacific Islander	5 (0.9%)
White	488 (85.8%)
Other	20 (3.5%)
Not answered	14 (2.5%)
Ethnicity	
Hispanic or Latino	36 (6.3%)
Not Hispanic or Latino	413 (72.6%)
Other	91 (16.0%)
Not answered	29 (5.1%)
Education	
Did not complete high school	21 (3.7%)
High school degree or equivalent (e.g. GED)	95 (16.7%)
Some college but no degree	131 (23.0%)
Associate degree (2 year college degree)	83 (14.6%)
Bachelor degree (4 year college degree)	145 (25.5%)
Graduate degree	80 (14.1%)
Not answered	14 (2.5%)
Work outside of home	
Yes	343 (60.3%)
No	213 (37.4%)
Not answered	13 (2.3%)
First person in family with NF	
Yes	368 (64.7%)
No	188 (33.0%)
Not answered	13 (2.3%)



How much does each FEATURE of your **raised** cutaneous neurofibromas bother you (physically, cosmetically, or emotionally)?



What is it about the cNF feature(s) that bother you?

Major Themes	Representative Quotes	N (%)
Psychological Health	"I am able to still carry on my day to day activities. I hate looking at myself in a mirror, am extremely self-conscious about my appearance and what others think of me... even my husband."	180 (73.8%)
Psychological (anxiety, depression, suicide)		
Appearance		
Self-esteem		
Feel the need to conceal oneself (clothes, makeup)		
Loss of "normal" life		
Embarrassment		
Physical Health	"...Although most of mine are quite small, they are in awkward places that rub, cause bleeding and extreme itching."	121 (49.6%)
Physical (pain, itch, bleeding)		
Progressive nature of NF1		
cNF location		
Cancer concerns		
Social Wellbeing	"It's been difficult to have and explore intimate relationships with men because of my body's appearance. I have been rejected because of the way my body looks..."	81 (33.2%)
Hurtful comments/stares		
Social isolation		
Family planning/Inheritance considerations		
Intimacy issues		

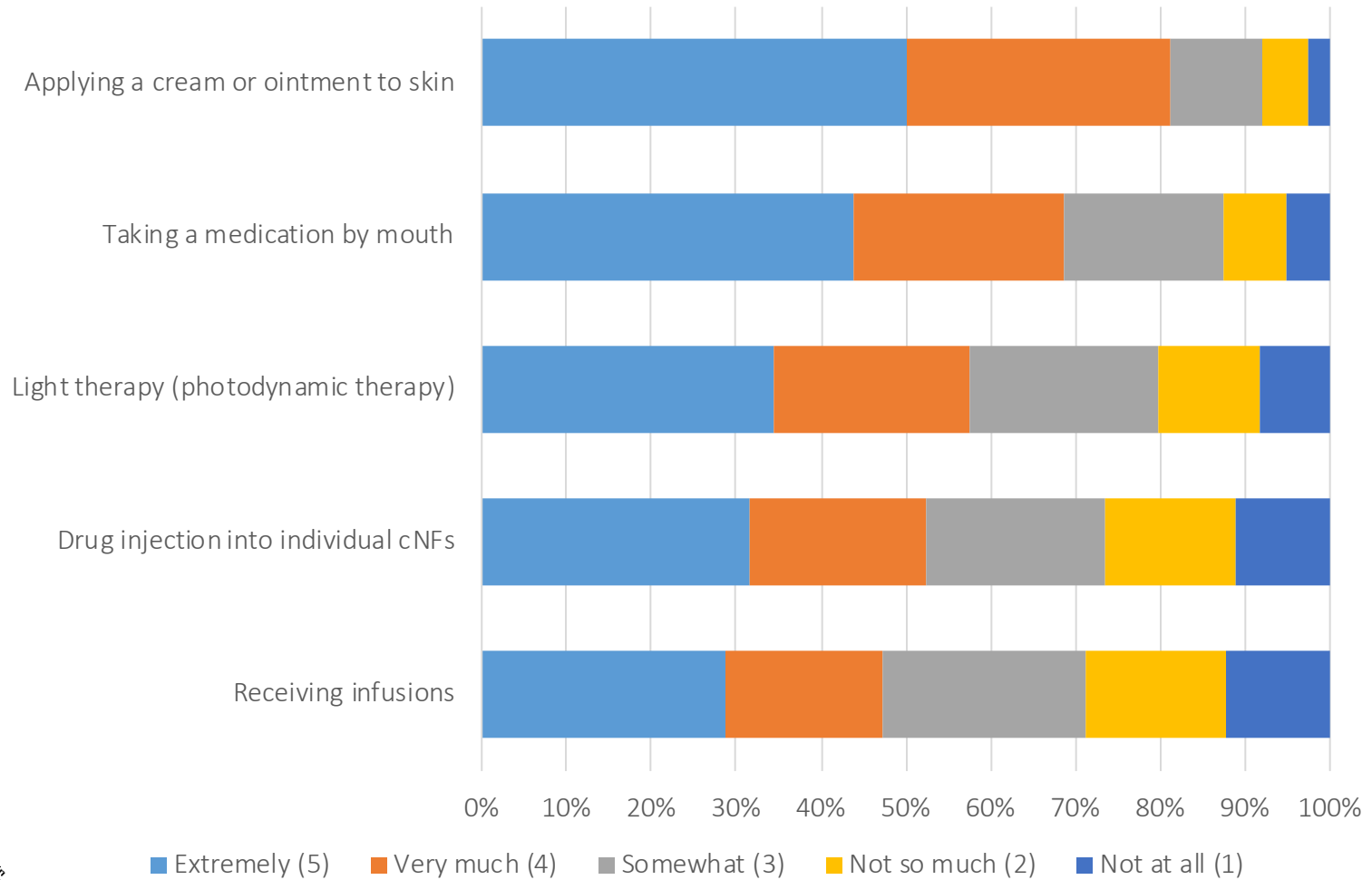


Recommendation #1

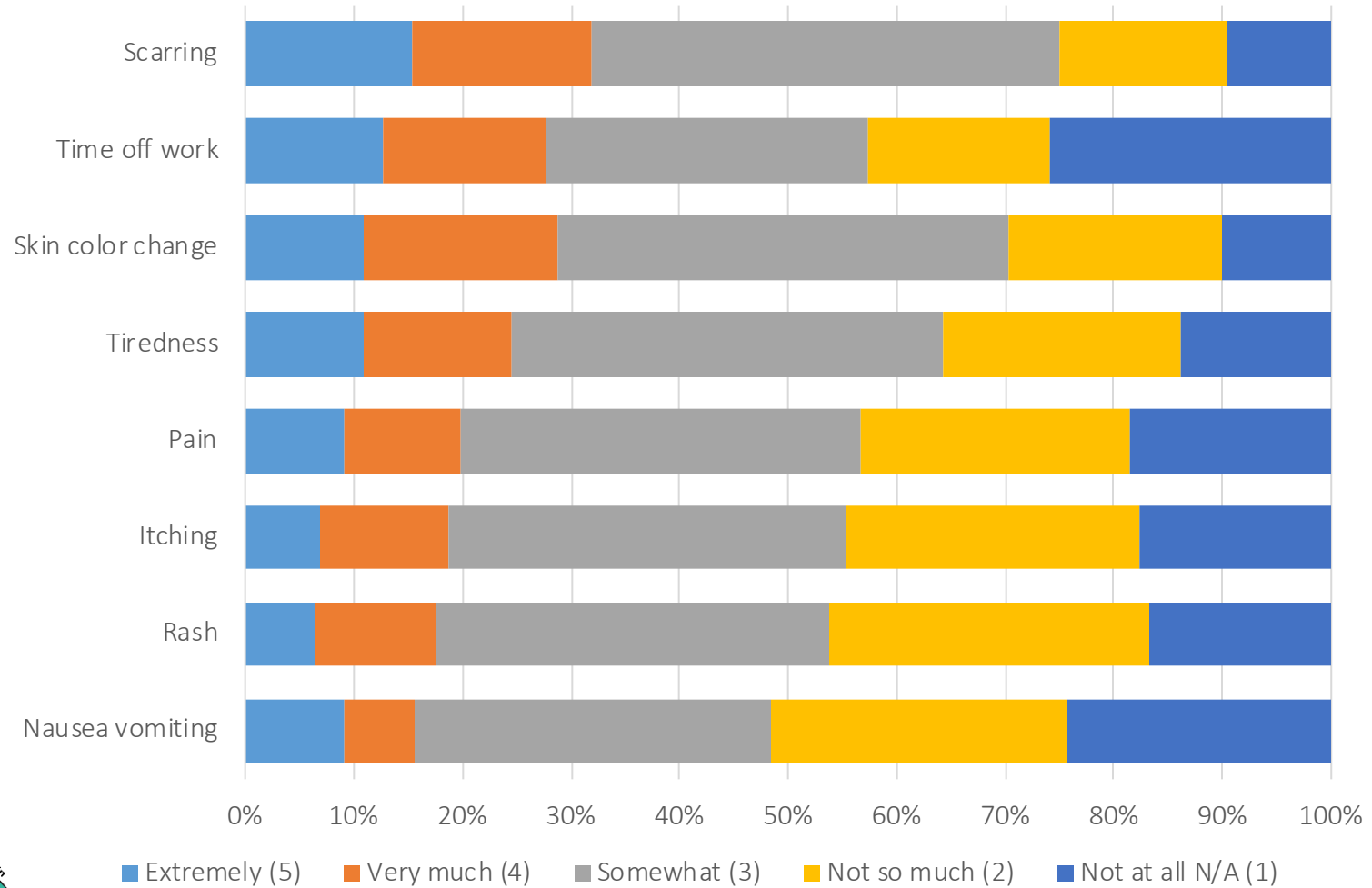
- Identification of, or development of, a quality of life index that captures the burden of disease and will be sensitive to change of cNFs



Assuming equal effectiveness and safety, what is your willingness to try these EXPERIMENTAL treatments for cutaneous neurofibromas?



What side effects are you willing to risk for treatment of your cutaneous neurofibromas even if they may grow back later?

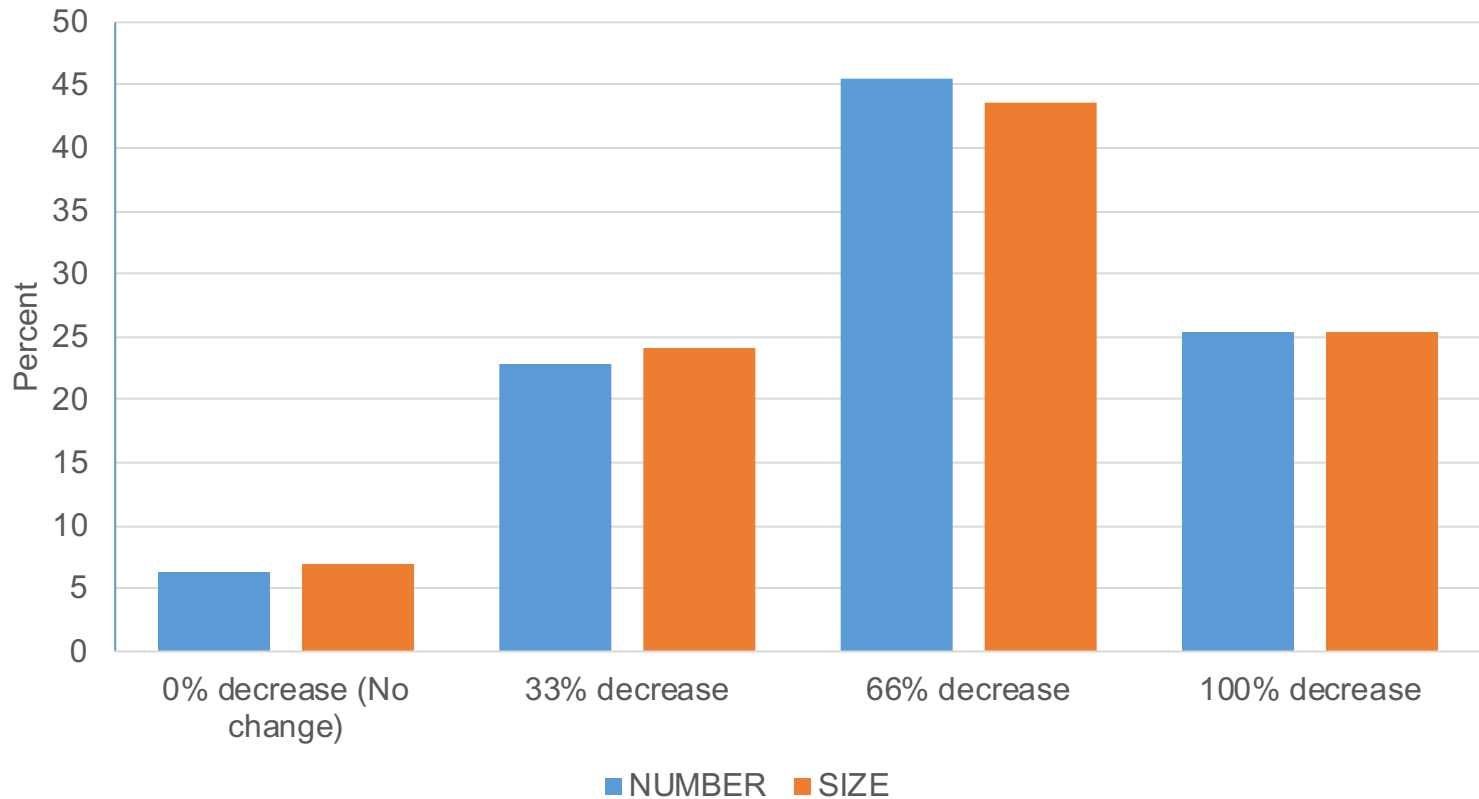


Recommendation #2

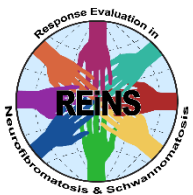
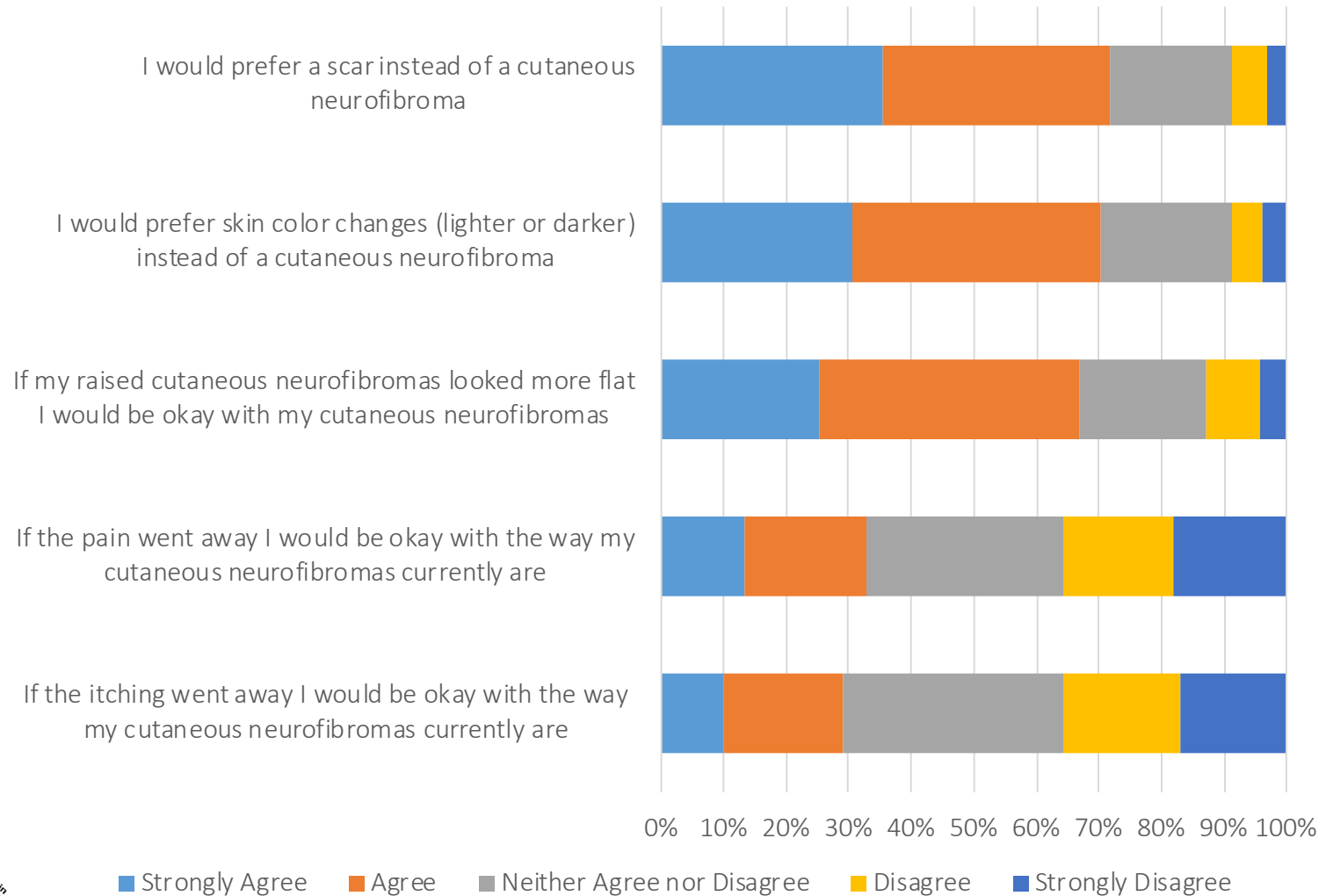
- Topical formulations may be best suited as systemic side effects would be less likely to occur and participants prefer topicals to systemic treatments



When thinking about the raised cutaneous neurofibromas that bother you the most, what is the minimum decrease that would be acceptable to you after participating in a treatment trial?



How do you agree with the following statements?



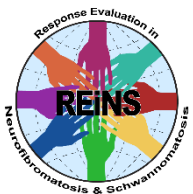
Recommendation #3

- When measuring response to therapy, the goal should not be 100% clearance of a cNF and the outcome measurement tools should be tailored to demonstrate a meaningful response, even if there is residual tumor or skin change



What might prevent you from participating in a clinical trial to treat your cutaneous neurofibromas?

Major Themes	N (%)
Cost	135 (77.1%)
Financial	
Travel	
Time commitment	
Insurance concerns	
Trial-specific Concerns	46 (26.3%)
Side effects	
Comorbidities	
Eligibility concerns	
Previous negative experience	
No proven efficacy	
Distrust	12 (6.7%)
Lack of Trial Awareness	
Current Life Situation	11 (6.3%)
Lack of support	
Poor health	
Pregnancy/family planning/young children	



Recommendation #4

- Reduce barriers to participating in cNF clinical trials



Recommendations for future cNF trials

1. Identification of, or development of, a quality of life index that captures the burden of disease and will be sensitive to change of cNFs
2. Topical vs. oral formulation of medication
3. When measuring response to therapy, the goal should not be 100% clearance of a cNF
4. Reduce barriers to participating in cNF clinical trials



Acknowledgements

- REiNS cNF Working Group
- REiNS Patient Representatives!
- UAB Biostatistics
 - Peng Li, PhD
- Funding
 - Francis S. Collins Award



THE UNIVERSITY OF
ALABAMA AT BIRMINGHAM

Knowledge that will change your world



Neurofibromatosis
Therapeutic Acceleration Program
at Johns Hopkins

