

Cutaneous Neurofibromas

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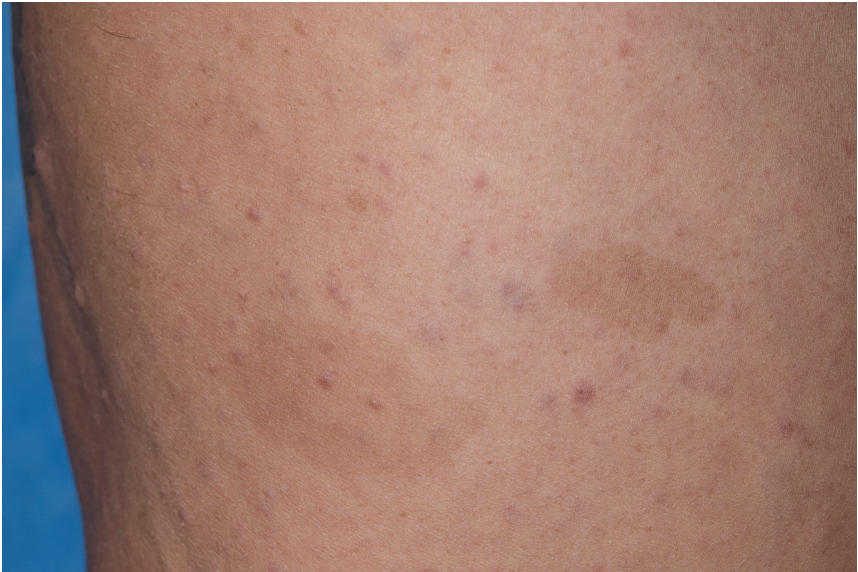
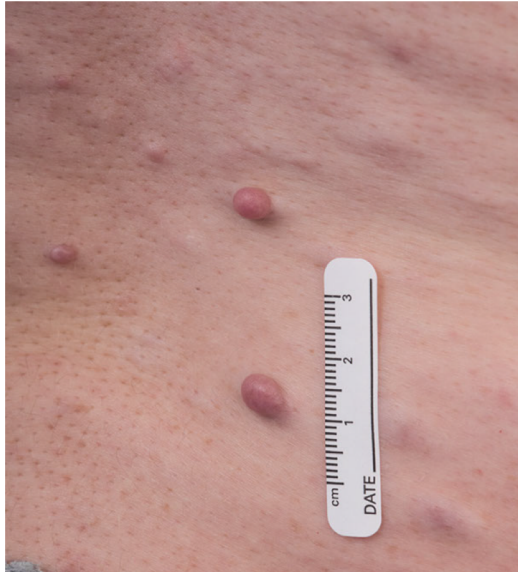
Response Evaluation In Neurofibromatosis Schwannomatosis
INTERNATIONAL COLLABORATION

Mission

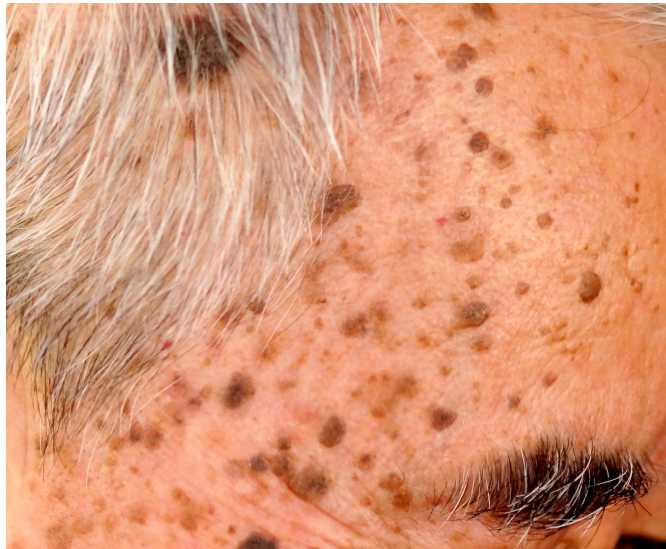
- Identification of research priorities for cNFs
- Development of recommendations for clinical trials for cNFs
 - Clinical trial endpoints
 - Clinical trial outcome measures
 - Clinical trial designs



Cutaneous Neurofibromas (cNFs)



NOT cutaneous neurofibromas



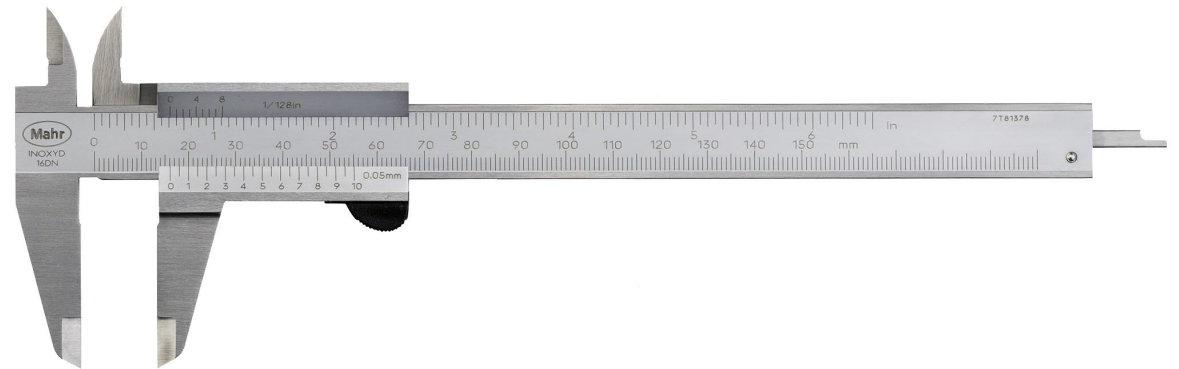
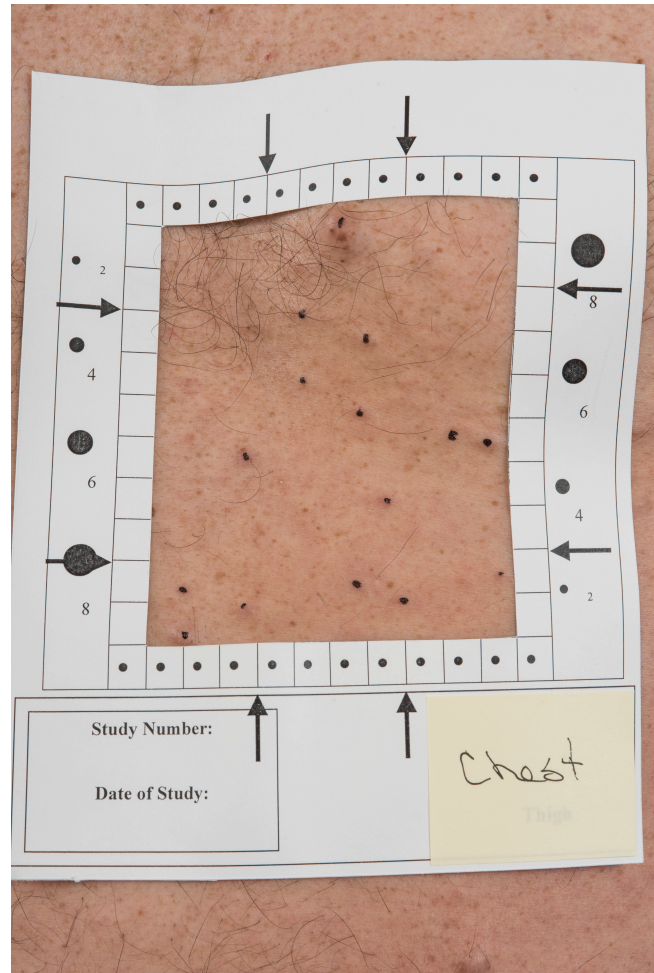
End point in a clinical trial for cNFs: The primary outcome that is being measured in an interventional trial

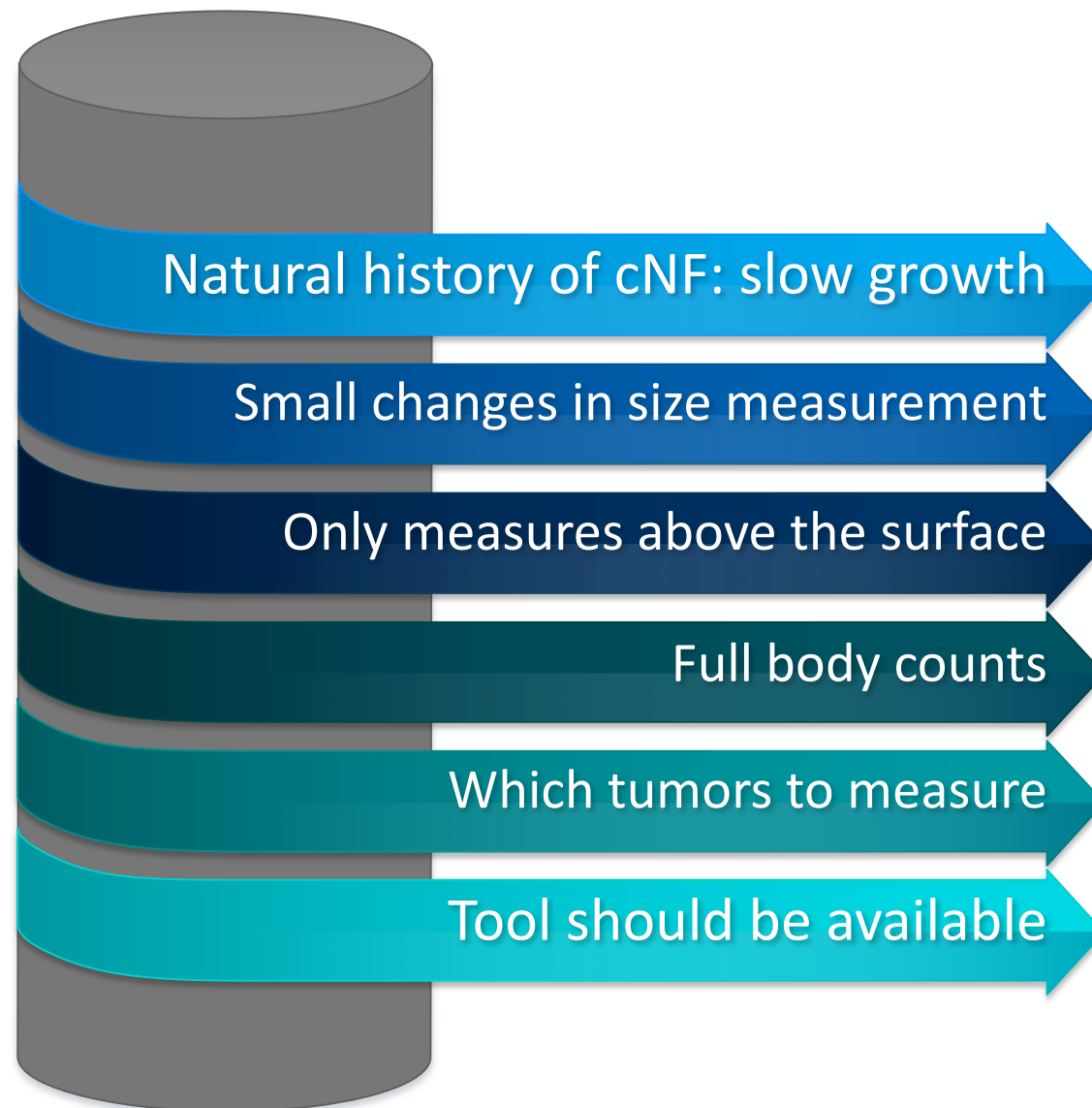
Number
of cNFs

WHAT IS MEANINGFUL CHANGE?

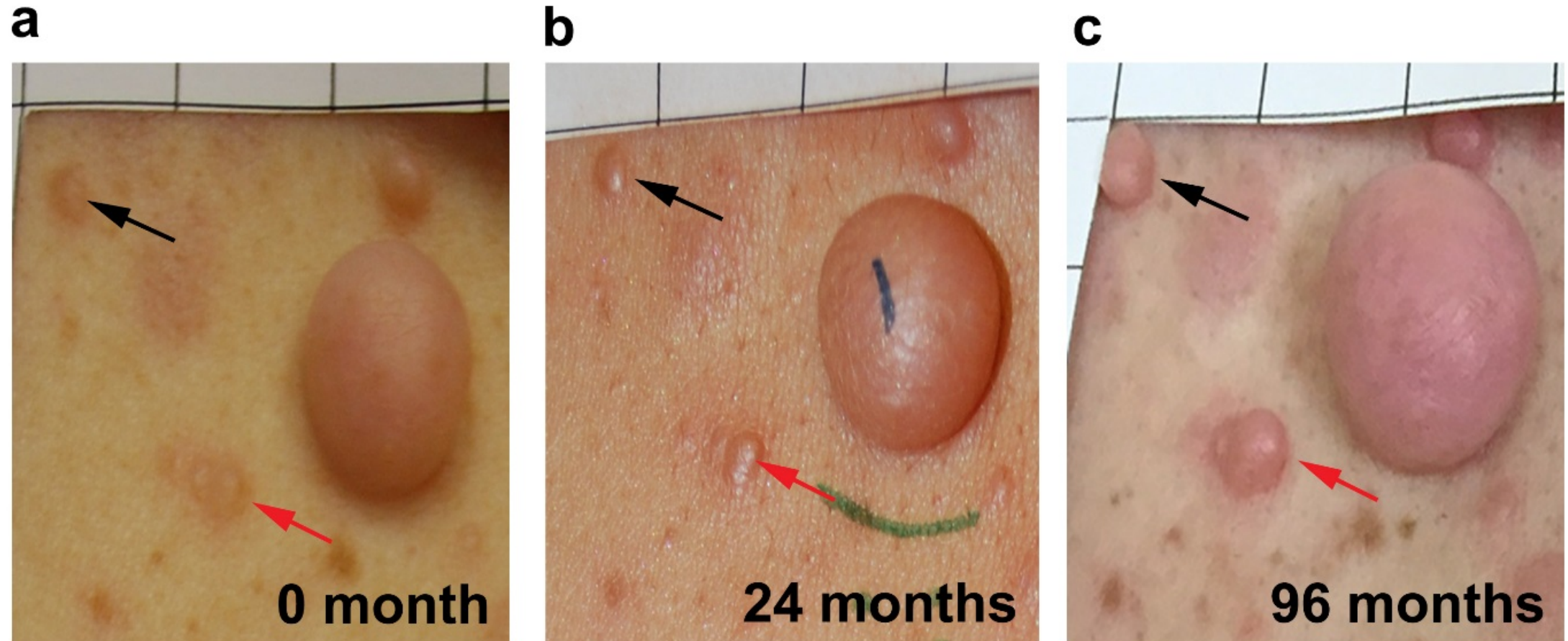
Softness
of cNFs

Current tools to measure change in size and number as end points





Visualization of cNF growth over 96 months



Patient Views Regarding Cutaneous Neurofibromas and Treatment

REiNS cNF working group

cNF patient surveys

How do patients view their cNFs?

Location

Size

Symptoms

Number

What do patients think about treatment options?

Result

Treatment type

Treatment length

Side effects

cNF patient surveys

Phase 1

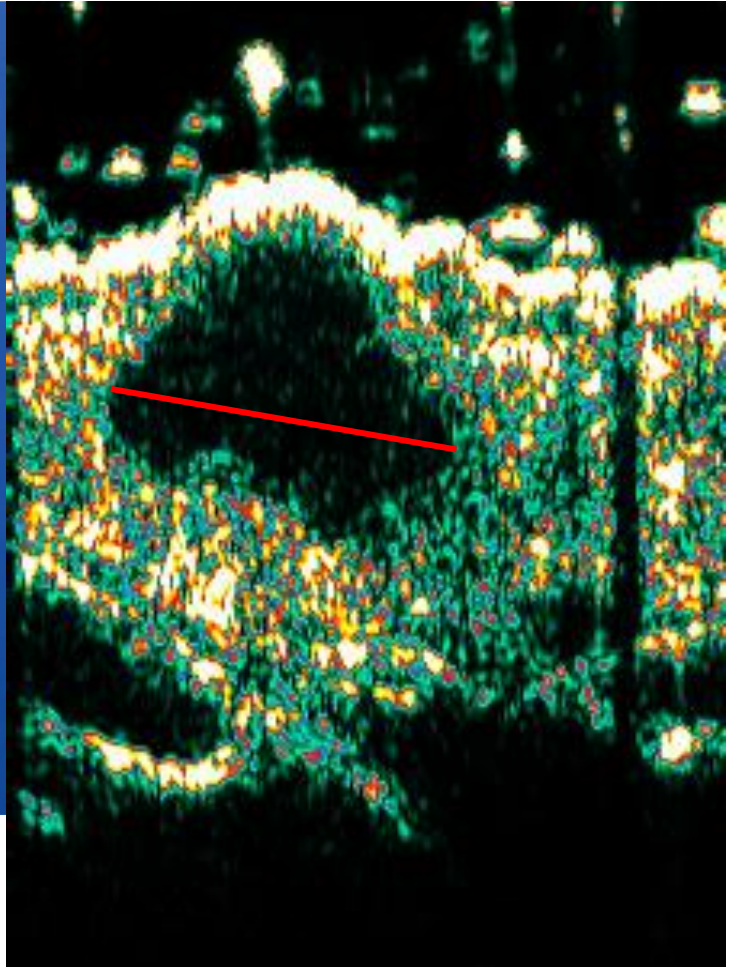
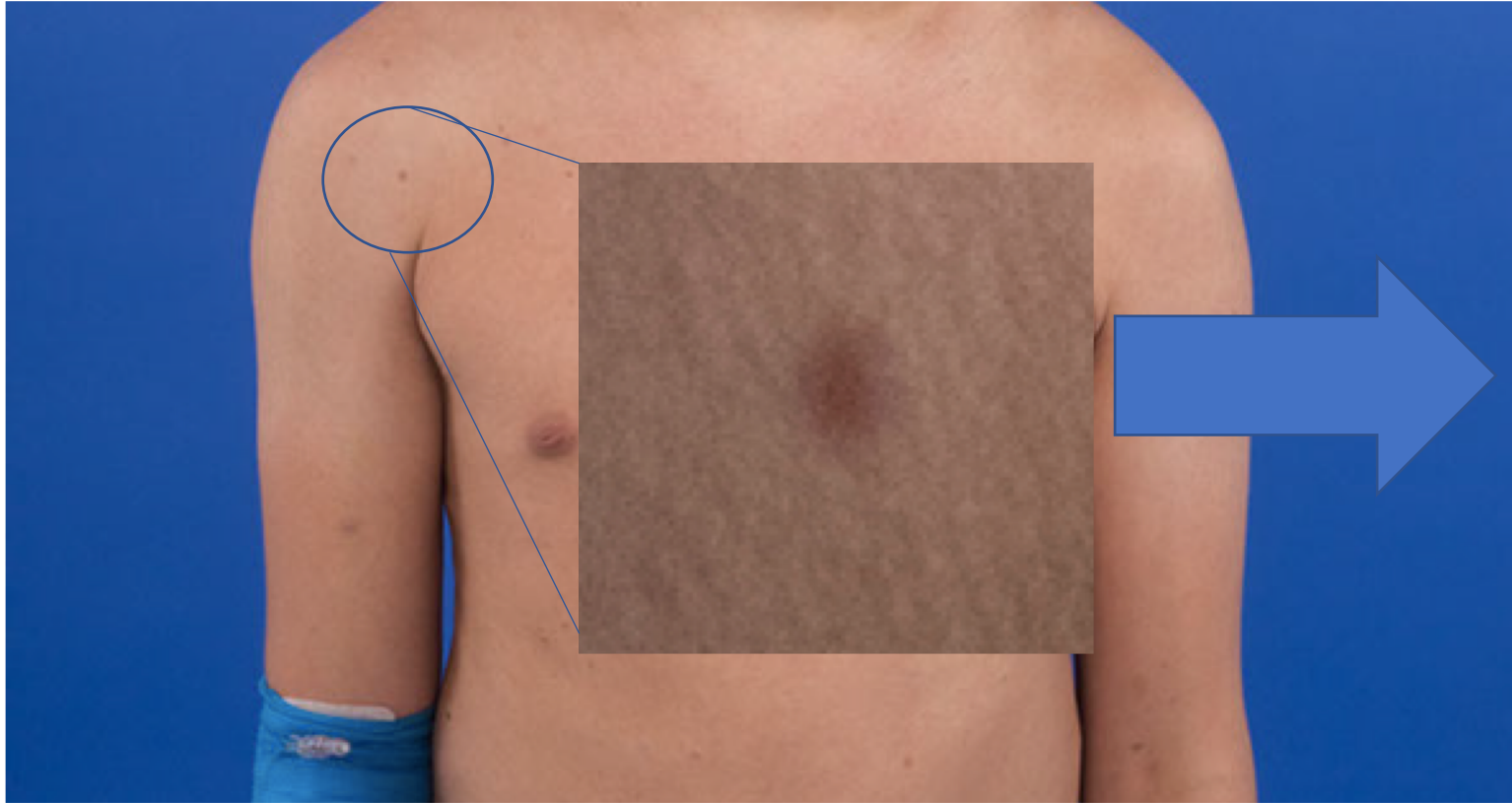
- Adults with cNFs survey
- Survey to be sent via CTF registry summer 2018

Phase2

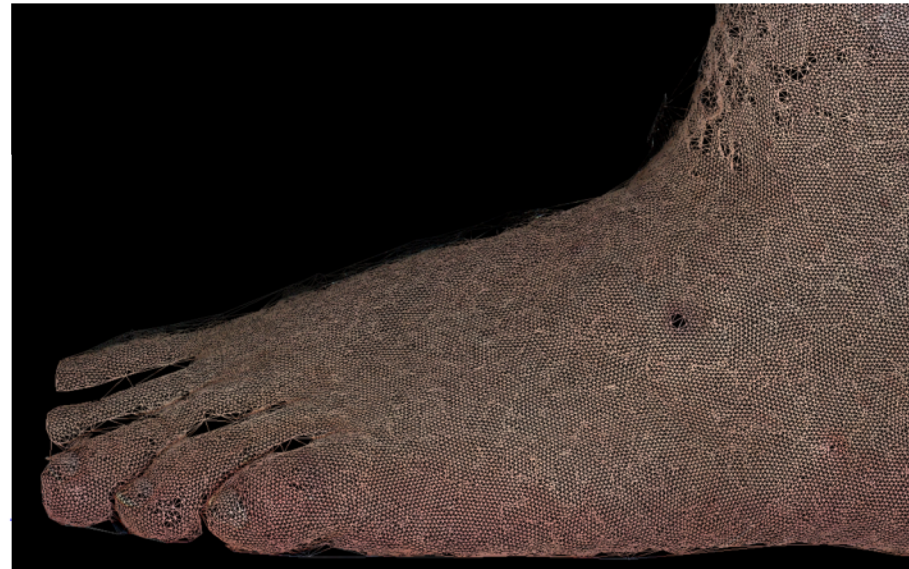
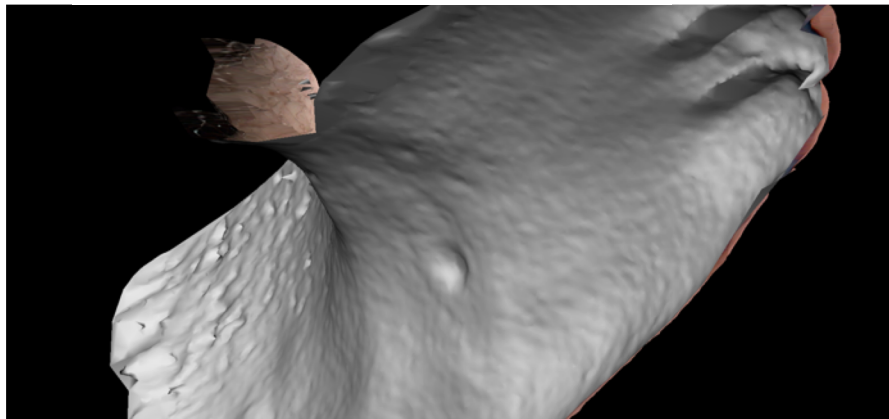
- Paired adolescent (12-17) and caretaker survey
- Survey to be sent via CTF registry fall 2018

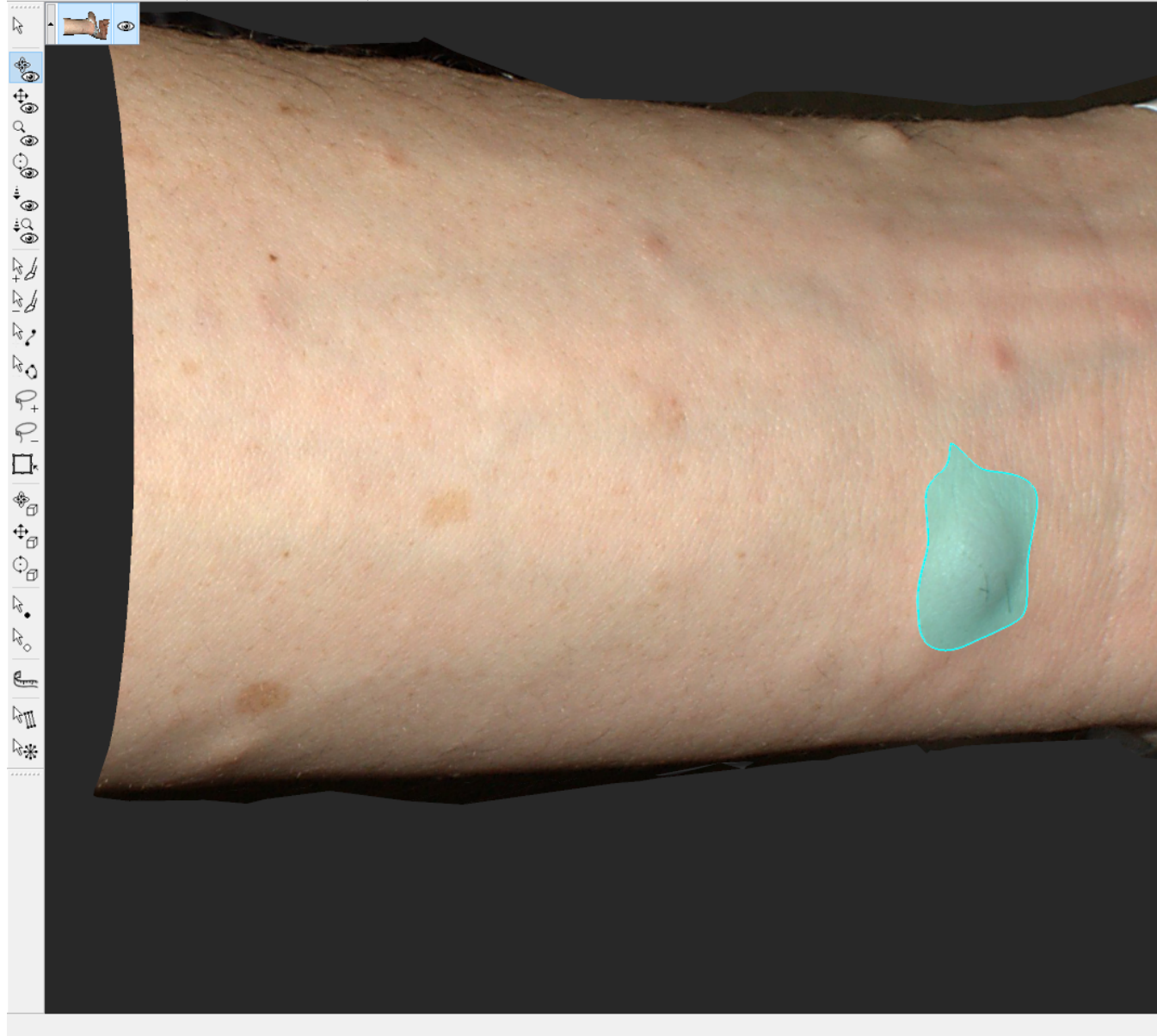
Proposed end point measurement tools

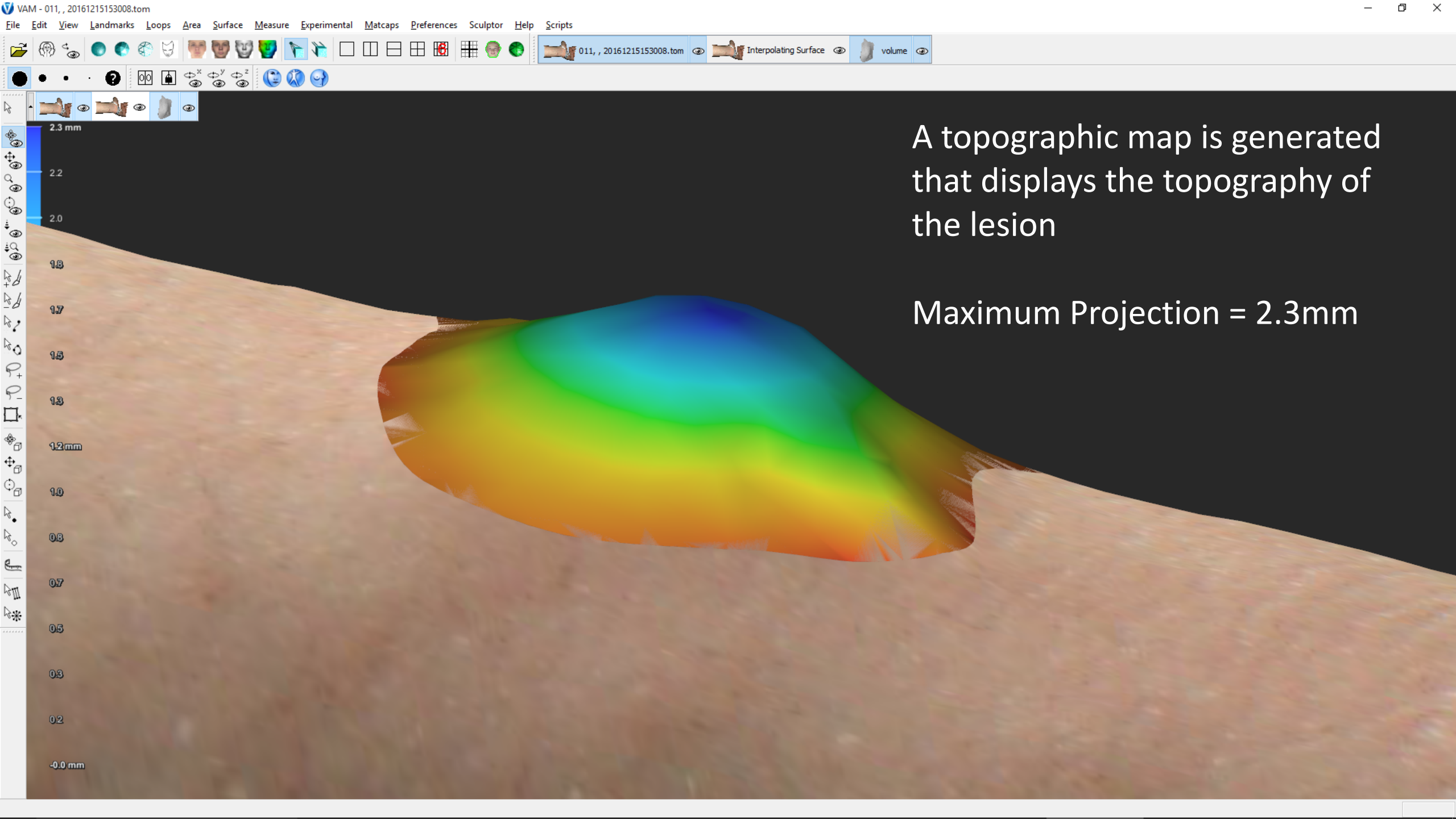




Volumetric photography to quantify the tumor volume

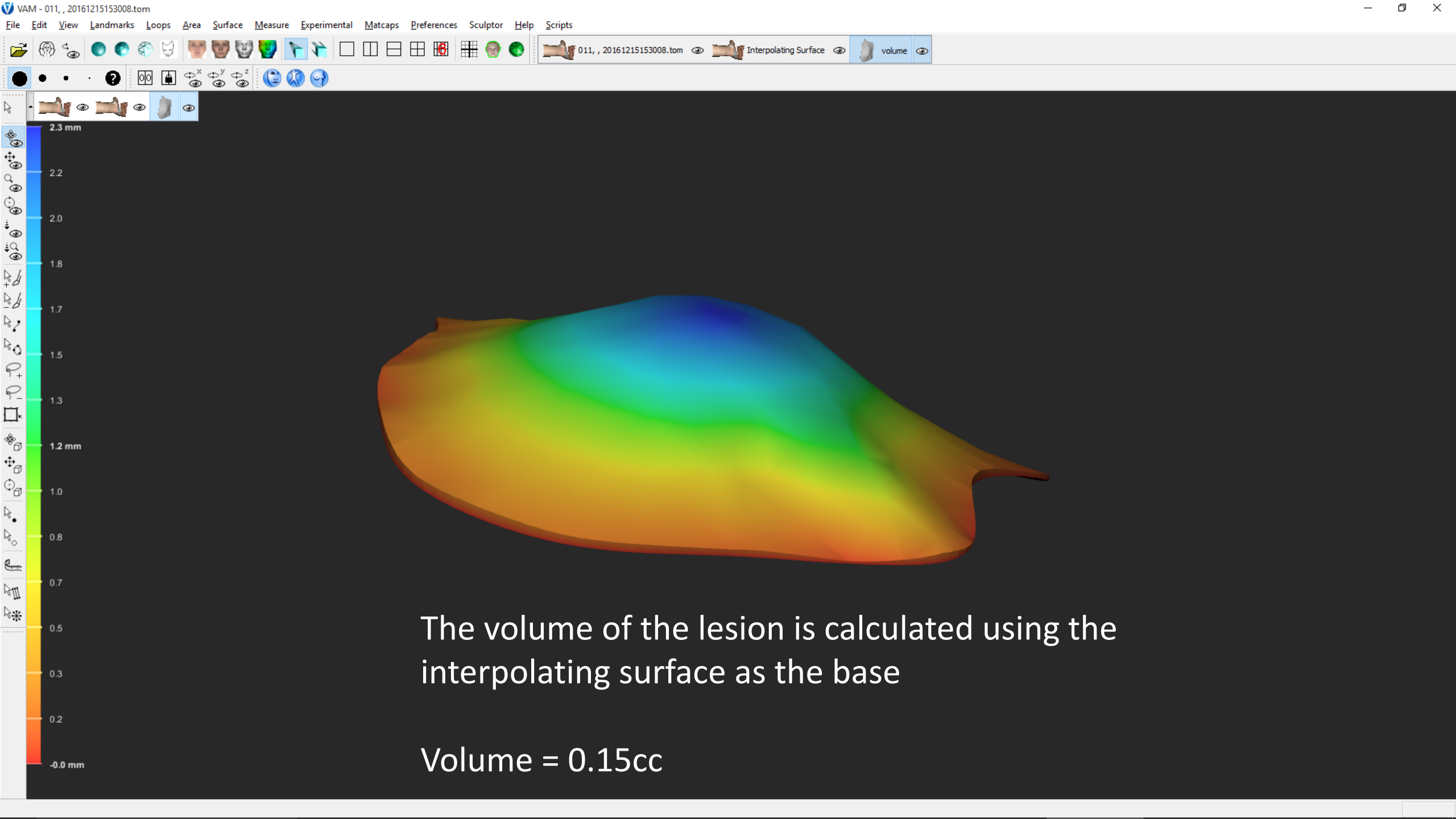






A topographic map is generated that displays the topography of the lesion

Maximum Projection = 2.3mm



The volume of the lesion is calculated using the interpolating surface as the base

Volume = 0.15cc

Pilot study of the MEK1/2
inhibitor selumetinib targeting
cNFs

TIMELINE

1st VISIT

Measure cNFs
Sample a cNF
Patient survey
Blood draw

BY 5th MONTH

Sample a cNF
Blood draw

MONTHLY

Measure cNFs
Patient survey

AT END

Measure cNFs
Sample a cNF
Patient survey

Sites: UAB and NIH

24 months of treatment

Adults aged 18 and over

Acknowledgements

- Patient representatives in the REiNS cutaneous neurofibroma group
 - Gregg Erickson
 - Sarah Adsit
 - Claas Röhl
- Pam Wolters, Patient reported outcome group
- Children's Tumor Foundation

