

# Exploring quality of life in people with Niemann-Pick disease type C

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## Background

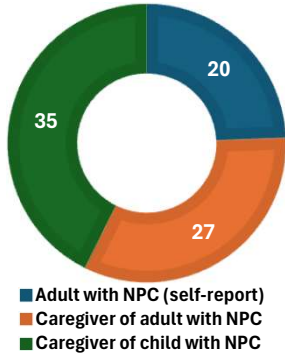
- NPC has a profound effect on the quality of life of both patients and their caregivers
- Specific questionnaires were created to capture symptom burden and QoL in adults and children with NPC
- However, these questionnaires need to be tested to ensure they are fit for purpose
- A survey is being run to collect data on these instruments

## NPC survey

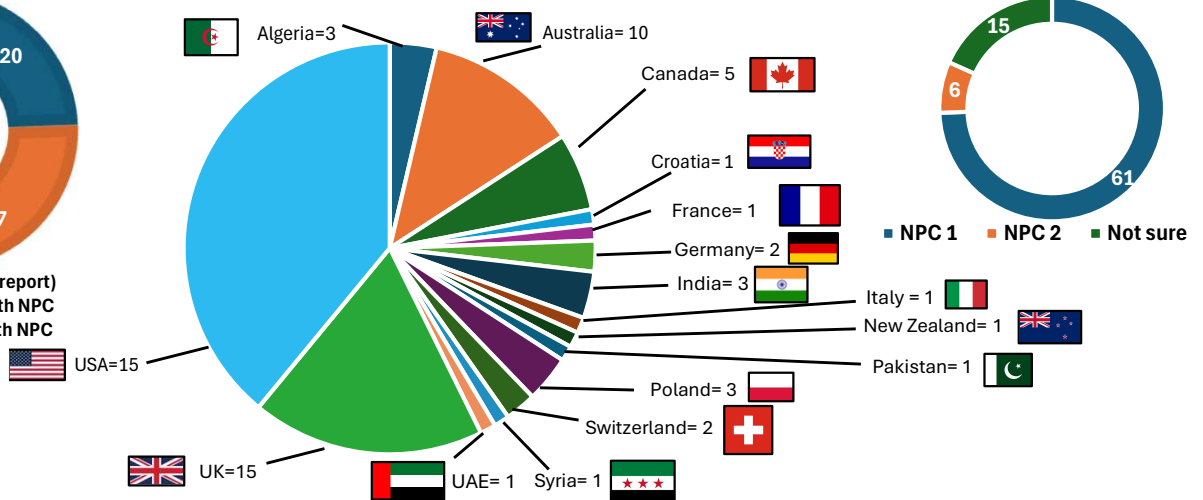
- Launched in January 2025- **ongoing!**
- Eligible participants include people with NPC over 18 years of age and caregivers of people with NPC (of any age)
- Collecting data on a variety of outcomes, including NPC QoL, general QoL, symptom severity, unmet need and caregiver QoL

## Summary of survey participants so far

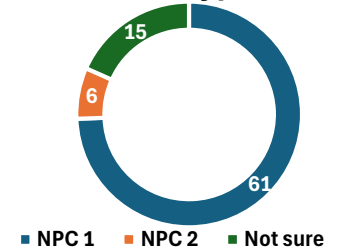
### Participant cohort



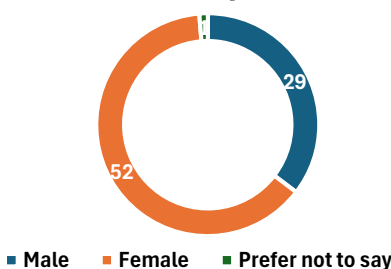
### Participant country of residence



### NPC type

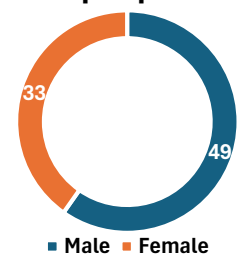


### Gender of respondents



Number of participants	82
Average age of participants	45.4 years
Average age of adults with NPC	36.8 years
Average age of children with NPC	9.8 years

### Gender of people with NPC



## Preliminary results

- The adult NPC QoL questionnaire shows a positive relationship with an existing QoL questionnaire, the short form-36
- Suggests the adult NPC QoL questionnaire is accurately capturing QoL
- No results for disease severity or QoL in children with NPC- need more data!

## What's next?

- We are continuing to collect data in English-speaking countries
- You can still take part in the survey by scanning the QR code below!
- Later this year, we will translate the survey into multiple languages to expand the study beyond English-speaking countries



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**Make sure your voice is heard- scan the QR code to take part in the survey!**

