PATIENT ENGAGEMENT IN GUIDELINE DEVELOPMENT

EPAG Building Good Practices Webinar
31 01 2022

Welcome!

We will be starting shortly…

- Mute your microphones
- Use chat box for Q&A and comments
- Raise your hand if you wish to speak
- If you experience technical problems, try logging out from zoom and logging back

This meeting is being recorded. By continuing to be in the meeting, you are consenting to be recorded and to have the recording available in EURORDIS YouTube channel. Your name will not be displayed in the recording.

EURORDIS RARE DISEASES EUROPE

EURORDIS.ORG

ePAG Building Good Practices

Patient Engagement in Guideline Development

31 01 2022

Agenda

16.00-16.05 - Welcome & Introduction

16.05-16.15 - Summary of PE in Guideline Development

16.15-16.30 - ERN CRANIO: Methodology and Patient Engagement - a Learning Curve

16.30-17.40 - *Questions & Answers*

16.40-16.55 - GENTURIS: Experience with the NF1 Tumour Management Guideline

16.55-17.25 - *Questions & Answers*

17.25-17.30 - Wrap-up

EURORDIS.ORG

Speakers

Gareth Davies

- Executive Director of the European Cleft Organisation
- ePAG advocate in ERN CRANIO

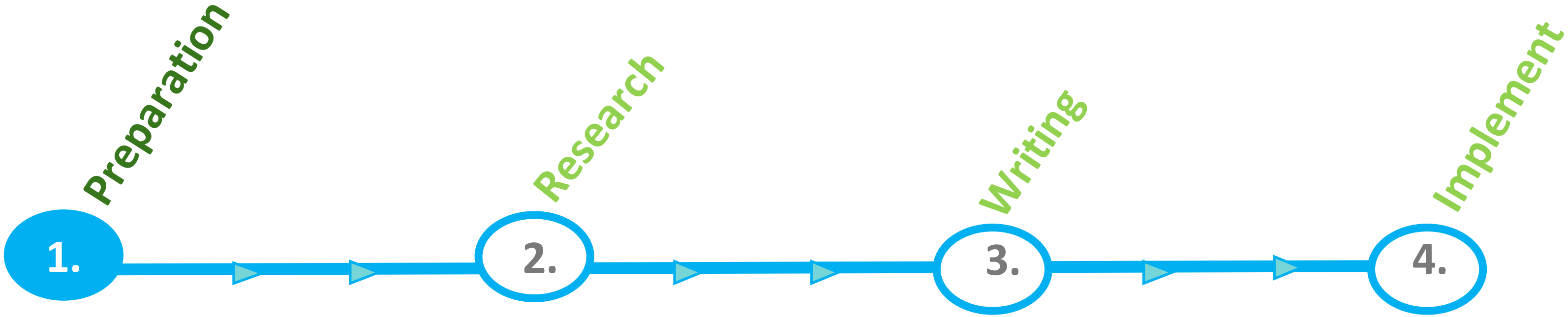
Claas Roehl

- President of NF Kinder
- ePAG advocate in GENTURIS

Overview of PE in CPG Development

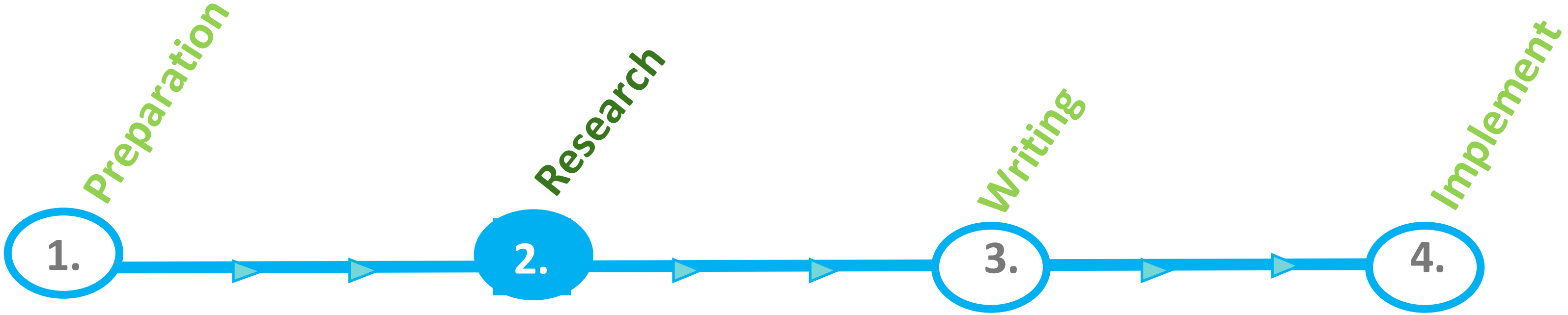
1. Preparation
2. Research
3. Writing
4. Implement

Overview of PE in CPG Development



-Patients play a critical role in this stage!
-**Recruit** patients to the Guideline Development Group (GDG)
- Get wider patient perspectives via a **Patient Advisory Group**
- Provide 'patient input training' to patients
- **Define the guideline scope** e.g. priority setting survey
-**Formulate clinical questions** e.g. PICO Questions
- Importance of outcomes from the patient perspective

Overview of PE in CPG Development

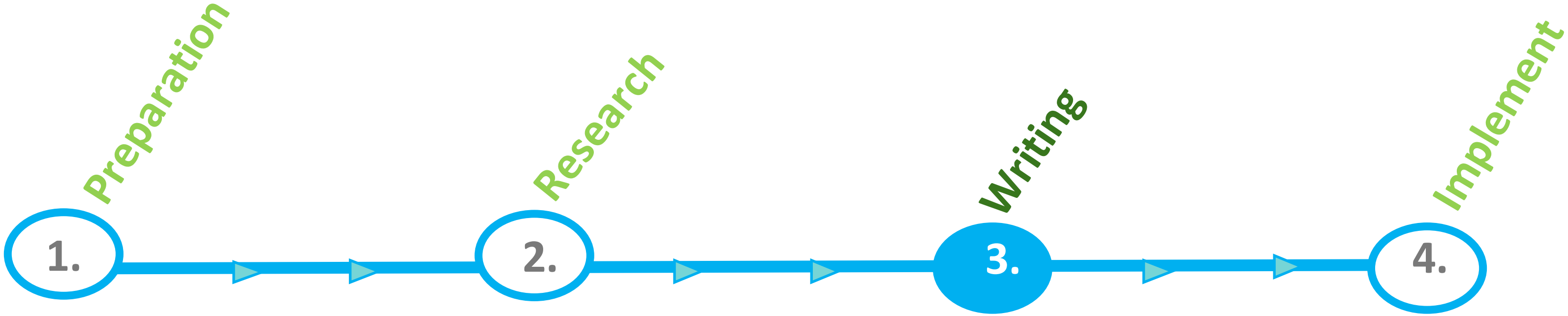


1. Preparation
2. Research
3. Writing
4. Implement

-Patient role is limited in scientific literature research!

- Patients as part of GDG are involved in:
- **Literature Search** (Screening & Selecting relevant papers)
- **Appraisal & synthesis** of evidence
- **Consensus Building** (e.g. Delphi)

Overview of PE in CPG Development



1. Preparation
2. Research
3. Writing
4. Implement

-Patient input is important to identify evidence gaps!
-Consider if evidence **reflects important outcome** measures
- Identify gaps in evidence
-Highlight areas of **patient preferences & patient choice** -Ensure guideline is worded appropriately
- Be part of the external **review** group

Overview of PE in CPG Development

1. Preparation
2. Research
3. Writing
4. Implement

Patients play an important role!

-Develop **lay-persons versions**

-**Disseminate** guidelines

CRANIO ERN

ERN CRANIO guideline Robin Sequence

Methodology and patient involvement - a learning curve

European
Reference
Network
for rare or low prevalence
complex diseases

Network
Craniofacial anomalies
and ear, nose and throat
disorders (ERN CRANIO)

Aim and scope

The aim of this guideline is **to provide** healthcare professionals and patients (and their parents) with Robin Sequence **an overview of the optimal care** concerning the various, multidisciplinary, aspects of Robin Sequence and to come with **recommendations to improve health outcomes and organization of care.**

Target population:

Healthcare professionals &

Patients and their families

ERN CRANIO expert centres RS

ERN CRANIO expert centres Vall d’Hebron, Barcelona Erasmus MC Rotterdam UMC Utrecht Karolinska Hospital San Bortolo Hospital Vicenza Santa Maria Hospital, Lisbon Uppsala University Hospital San Gerardo Hospital , Monza Hospital 12 de Octubre, Madrid Hôpital Universitaire Necker, Paris Helsinki University Hospital

Methodological support
Qualicura (NL)

European Reference Network
for rare or low prevalence complex diseases

Network
Craniofacial anomalies and ear, nose and throat disorders (ERN CRANIO)

Coordination

Erasmus MC *as* ERN CRANIO coordinating centre

- Organization of project meetings
- Coordination of project activities
- Liaise with Qualicura (methodological partner)
- Monitor progress and timelines
- Financial management

<u>Timeline</u>

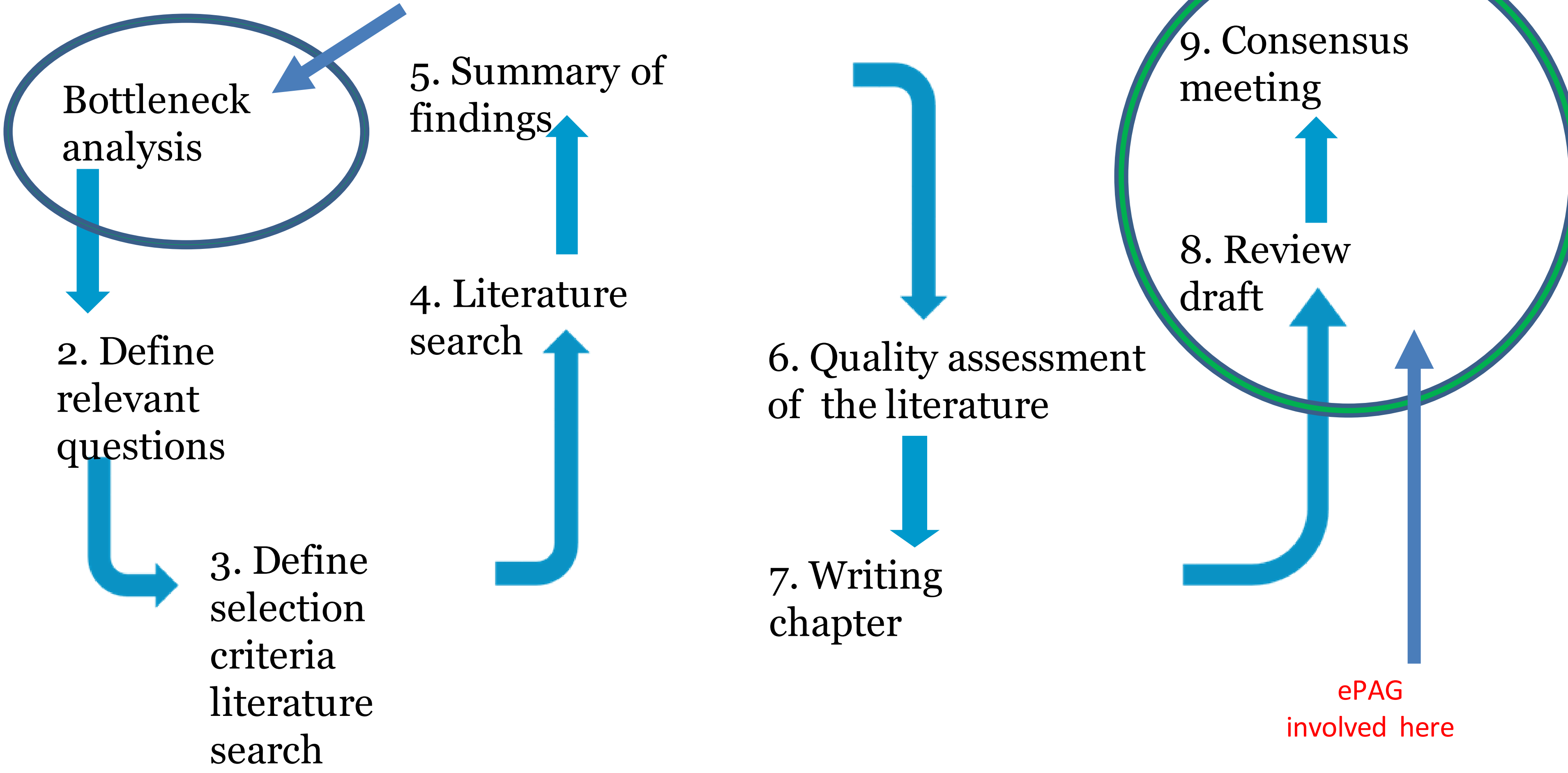
Start February 2021 - End February 2022

July 2021 - meeting with ERN Coordinator and Managers

To provide an opportunity for us to learn more about what being done to include patient representation in the guideline development

as an opportunity to explore ways in which this representation can be better organized and increased

Process



Patient survey here, input from specialist nursing

Bottleneck analysis

2. Define relevant questions

3. Define selection criteria literature search

4. Literature search

5. Summary of findings

6. Quality assessment of the literature

7. Writing chapter

8. Review draft

9. Consensus meeting

ePAG involved here

Patient engagement steps in red

Feb '21

- KICK OFF meeting
- Assign multidisciplinary steering group

Feb-Apr '21

- Identify patients' bottlenecks (survey)
- Define questions and outcomes
- Literature search

Apr '21

- *Online* meeting Steering Group
- Results bottleneck analysis
- Review concept outline RS guideline

Apr - Nov '21

- Write draft chapters guideline
- Review draft chapters guideline (Steering Group)
- *Online* meetings to discuss feedback / progress
- Nov '21 -- Steering Group consensus meeting

Feb '22

- Final version ERN CRANIO guideline RS
- Start patient version & translations

European Reference Network
for rare or low prevalence complex diseases

Network
Craniofacial anomalies and ear, nose and throat disorders (ERN CRANIO)

Guideline Chapters

1. General Introduction
2. Methodology
3. Genetics
4. Diagnostic criteria
5. Prenatal and counseling
6. Breathing & airway
7. Feeding & growth
8. Speech
9. Dentofacial
10. Psychosocial
11. Non-surgical management
12. Surgical management
13. Surgical treatment of palate-related problems
14. Quality of Life
15. Organization of Care

Essential patient perspective input provided

European Reference Network
for rare or low prevalence complex diseases

Network
Craniofacial anomalies and ear, nose and throat disorders (ERN CRANIO)

Summary patient input ★

to advise on the guideline scope, target population, clinical questions – all relevant to patient?

ensure literature review includes patient centred care and reported patient outcomes where available

★ survey the patient community where there are gaps in evidence

to provide the patient's opinions / views in balanced in terms of the benefit and harm of the recommendations

★ to be involved in formal consensus finding

★ to develop a lay-persons guide or information of the guideline

Next steps to finalize

- ☐ The RS guideline manuscript has been sent to all ERN CRANIO members, incl patient representatives for **review**
- ☐ Final version RS guideline to be published
- ☐ Local implementation in ERN CRANIO centres
- ☐ Patient version in all EU languages

Summary

- Dialogue slow to start but now constructive – this will inform development of other guidelines

- Despite not having patients (and/or ePAG reps) as part of the core guideline group, the importance of patient experience was recognised as an essential part of the process – the patient surveys generated a large number of responses from a wide number of locations. These were from expert centres but importantly patients reflected on their negative experiences before being referred to these centres

- Consensus meeting was attended by ePAG lead whose views and opinions in key areas were noted and he is included in continuing dialogue around revisions

- New ePAG terms and conditions will serve to formalise patient roles in ERNs and facilitate full engagement at all levels, and at all stages of processes

Q&A

GENTURIS

European
Reference
Network
for rare or low prevalence
complex diseases

Network
Genetic Tumour Risk
Syndromes (ERN GENTURIS)

NF1 Tumour Management Guideline

Claas Röhl
Patient representative, ERN GENTURIS

ePAG Building Good Practices,
31 January 2022

www.genturis.eu

NF1 Tumour Management Guideline Scope

Overall Scope	• The scope of this guideline is surveillance, follow-up and management of tumors in people with neurofibromatosis type 1.
Patient Population	• All individuals with NF1
Clinical Questions	• What clinical screening is beneficial for detecting tumours? What, when and how often. • What imaging screening (and surveillance) is beneficial for detecting tumours? • When a tumour is diagnosed, is the indication for surveillance different in NF1? What is content of surveillance (mode, interval)? • When a tumour is diagnosed, is the indication for treatment different in NF1? What is the indication for treatment in NF1? • Is treatment different in NF1? What NF1 specific treatment? • What psychosocial support do people with neurofibromatosis type 1 benefit from, specifically in living with the uncertainty of at risk tumours or in the management of a diagnosed tumour.

Tumour Types

Breast cancer

Gastrointestinal stromal tumors (GIST)

Juvenile myelomonocytic leukemia (JMML)

Orbital/ Periorbital Plexiform Neurofibromas

Cutaneous neurofibroma

Optic pathway glioma

Glomus tumours of the digits

Plexiform neurofibroma

Phaeochromocytoma

Malignant peripheral nerve sheath tumour (MPNST) & atypical neurofibromatous neoplasm with uncertain biologic potential (ANNUBP)

Non-optic glioma in children

Non-optic glioma in adults

Guideline Group

Delphi Process (n=>120)

7 Member States + 9 Member States + 7 Member States = 19 Member States

Core Writing Group (n=12)

Guideline Group (n=21)

Patient Advisory Group (n=7)

NF1 Guideline Group

	Speciality	**Member States**
1. Core Writing Group	Pediatrician, Clinical Geneticist, Dermatologist, Neurologist, Neuro-oncologist, Radiologist, Maxillofacial Surgeon, Patient Representative.	12 Leads from 7 Member States NL, UK, BE, FI, ES, AT and DE.
2. Guideline Group	Neuropsychologist, Adult Oncologists, Dermatologist, Sacroma Surgeon, Ophthalmologist, Neurosurgeon, Plastic Surgeon, Pediatrician, Clinical Geneticist, Neurologist, Neuro-oncologist, Radiologist, Maxillofacial Surgeon and Patient Representatives.	21 Leads from 9 Member States NL, UK, BE, ES, AT, IT, EL, DK and DE. Plus US advisors (x2)
3. Patient Advisory Group	Patient Representatives.	7 Leads from 7 Member States AT, ES, BE, EL, DE, PT and UK.
4. Delphi Expert Panel	Paediatrician, Oncologist, Clinical Geneticist, Dermatologist, Neurologist, Neurosurgeon, Gynaecologists, Psychologist, Psychiatrist, Ophthalmologist, Neuro-oncologist, Radiologist, Maxillofacial / Plastic Surgeon, Clinical Nurse, Patient Representative	>120 EU Experts from 19 Member States AT, PL, DE, FR, BE, NL, ES, EL, FI, IT, UK, SE, NO, DK, LT, CZ, SI, PT and EE.

Process

Spring 2020

1.

Preparation

- Convene Team
- Identify Topic
- Tools & Resources

Summer 2020

2.

Research

- Specify Topic
- Literature Search
- Grade Evidence

Autumn 2020

3.

Writing

- Draft Clear Recommendations
- Complete 1st Draft

2021

Consensus Building, writing

- 3 Round of questionnaires (spring – summer)
- Complete Final Version (autumn – winter)
- Review and approval

4.

Spring 2022

Implementation

- Editing
- Draft journal publication

Future Priority Areas for NF

1. **Endorsement of the new diagnostic guideline** for NF1, NF2 and Schwannomatosis (when this has been published)
2. Development of a **clinical pathway** for NF2 and Schwannomatosis
3. **Development of an NF2 guideline** (update on the ACCR and include the adult patients in the scope)
4. **Development of a guideline for other presentations for NF1** - e.g.: orthopaedic and neurocognitive issues

Q&A

EURORDIS
RARE DISEASES EUROPE

Patient involvement in development of clinical guidelines

Methods of engagement based on user experience of involvement in other clinical and service guidelines

Clinical Standards Advisory Group 1998 – government appointed body

Reconfiguration of cleft services in the UK

National Institute for Clinical Excellence, UK, 2008

Guideline on Otitis media with Effusion

European Committee for Standardisation (CEN), Brussels 2016

Early Care services for children born with cleft lip and palate

Central role

Involvement at every stage of the process, from start to finish

Patient representatives in the development committee

Patient roles

to advise on the guideline scope, target population, clinical questions – all relevant to patient?

ensure literature review includes patient centred care and reported patient outcomes where available

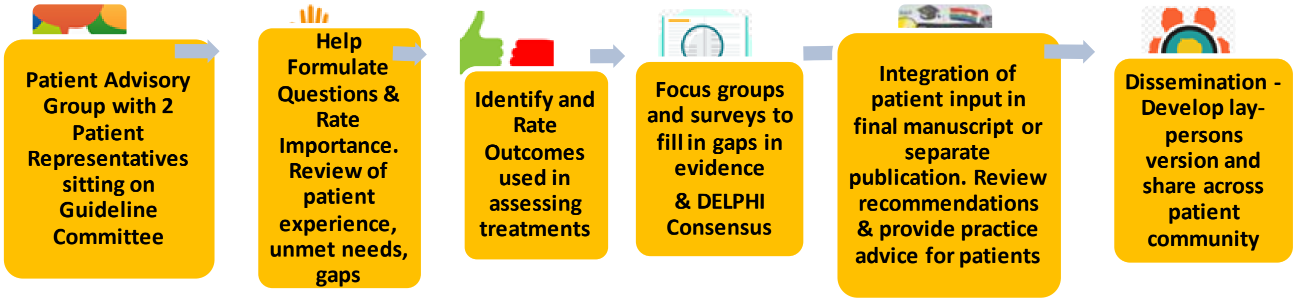
survey the patient community where there are gaps in evidence

to provide the patient’s opinions / views in balanced in terms of the benefit and harm of the recommendations

to be involved in formal consensus finding

to develop a lay-persons guide or information of the guideline

Patient input summary



Stage 1: Preparation

Patient Advisory Group with 2 Patient Representatives sitting on Guideline Committee

Stage 2: Research

Help Formulate Questions & Rate Importance. Review of patient experience, unmet needs, gaps

Identify and Rate Outcomes used in assessing treatments

Focus groups and surveys to fill in gaps in evidence

& DELPHI Consensus

Stage 3: Writing

Integration of patient input in final manuscript or separate publication. Review recommendations & provide practice advice for patients

Stage 4: Implementation

Dissemination - Develop lay-persons version and share across patient community

Wider input and dissemination

Patient reps on the guideline committee can be supported by a wider patient group

Representatives from different countries, with different experiences, bringing with them voices of other clinicians they are familiar with as potential external advisors

Opportunities for wider feedback and input

Ideas for wider patient input

Patient reps on Guidline committee supported by:

8 – 12 group of patients from different countries with different expereinces

Providing feedback and input to patient reps on Guideline committee