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"The patient is speaking": discovering the patient voice in ophthalmology

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ABSTRACT

Eye disease can be devastating. The most feared impact is sight loss, but in a number of ophthalmic conditions, there can be wide-ranging systemic, psychological, emotional and social effects of both the disease and its treatment. External tests of visual function, such as visual acuity, are inadequate to understand the overall impact of ophthalmic disease on a patient's functional vision or daily life. This can lead to a discordance between the patient's priorities and perspective on the one hand and the efforts of clinicians and other stakeholders on the other hand. In this review, we discuss how the patient is uniquely placed to understand the impact of the disease and can use that position to transform ophthalmic care at the individual and collective level, from research to care delivery. We highlight how the 'patient voice' can contribute to key areas, including priority setting in the research agenda, communicating the wide-ranging impact of disease and its treatment as assessed through qualitative research, identifying the outcome measures that matter to the patient through core outcome set development and reporting these outcomes through appropriate patient-reported outcome measures. We also consider the increasing power of the patient voice on health institutions, ranging from broadcasting an individual's experience of care he/she has received to patient societies influencing future health policy. Finally, we reflect on the challenges that need to be overcome for the patient voice to increasingly influence and improve the delivery of eye care in the future.

INTRODUCTION

In around the fifth century BC, Protagoras stated that 'Man is the measure of all things'. This early statement of 'relativism' affirmed the value and, it was argued, the 'truth' of an individual's perspective, even if it could not be measured objectively, irrespective of the extent to which it differed from others. In an era of evidence-based medicine, we prize external objectivity and would counter Protagoras with the less pithy, 'A sensitive and specific endpoint assessed by a precise and accurate instrument is the measure of all things'. Consequently, the current practice of medicine may try to reduce a patient's state—the physical, emotional, psychological, societal health and well-being—to a collection of external 'objective' measurements. The practical danger of this approach is that medicine may now impose health states on patients, rather than accepting the patient's own evaluation. Examples from the clinic abound. A doctor tells a patient, 'You must be better. Your visual acuity has improved', to which the patient responds, 'But I don't feel that I can see any better. I still can't drive, and these tablets you have given me make me feel sick all the time'. In many cases, the weight of expectation may even mean that the patient leaves the thought unspoken or even acquiesces with the clinician's perspective.

The 'patient voice' needs to be heard at an individual and at a collective level. Good medical practice may pride itself on being 'patient centred', but this is still often based on a paternalistic assessment by the clinician of 'what is best for the patient'.[1 2] We argue that greater recognition of the value of the patient voice will allow a reorientation of both medical research and clinical practice around the patient perspective and ensure that the priorities of researchers, clinicians, funders and policymakers are aligned to the needs and priorities of those who experience disease.[3] There is increasing evidence in other specialities, particularly in oncology, that adopting this approach in clinical care can bring demonstrable patient benefit and reduce healthcare costs.[4–10] In this narrative review, we will focus on those with eye disease, considering such patients and their relationship with the wider community as a case study. Through this review, we aim to show how, as a community, we need to value the patient voice and, in so doing, gather the collective experience and insights of patients to improve understanding of the disease; together with patients, we can assess the benefit and harm of treatments, provide feedback on the quality of care delivery and set the research agenda.

THE PATIENT VOICE: LEARNING TO LISTEN

The steady power shift from a paternalistic healthcare professional to an empowered patient is revolutionising the dynamics of the individual consultation. There is increasing acceptance that the patient voice must be heard and that patients are key decision makers in their care, with patients usually placing greater emphasis on the non-clinical aspects of treatment. This transformation is occurring in both 'top-down', and 'bottom-up' directions. The 'top-down' emphasis on the individual patient voice can be seen in a number of national initiatives. The UK government's 2010 white paper, 'Equity and excellence: Liberating the NHS', included the priority that 'shared decision making will become the norm: *no decision about me, without me*'.[11] In the USA, the Patient Protection and Affordable Care Act specifically

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highlights a need for patient centredness and includes initiatives such as a 'programme to facilitate shared decision making' and the establishing of a 'patient-centred outcomes research institute'.[12]

Patient-reported outcome and experience data are now collected routinely for some common procedures, and electronic media provides opportunities to instantly give feedback on the quality of care. Grass-roots developments include initiatives that transfer control of a patient's health record towards the patient. There is an interesting spectrum of practice encompassing those in which the care provider continues to own the record and control the level of access provided to the patient and initiatives such as the 'patient-knows-best' system, in which patients own the record and control the access given to their various health providers.[13] Although all such initiatives underline the movement of power from healthcare provider to individual patient, some are more about control than communication. While it is understood that power and communication are, in fact, indivisible, the focus of this review is primarily on the latter, emphasising the patient voice as it communicates experience and insight from the patients to those around them who seek to care for them, whether directly or indirectly. We will consider the practical ways in which the clinical and research communities can learn to listen to both the individual and collective patient voice, recognising the different ways it may be expressed and the insights that this may bring (table 1).

The patient's voice in setting the research agenda

The increasing influence of patients individually and collectively on research is seen at every level, with numerous initiatives to support patient and public involvement (PPI). Organisations such as the James Lind Alliance (JLA) help bring the patient voice to the research agenda, and priority-setting partnerships may define the ophthalmic research agenda for an institution, a funding body or even a whole country. For example, a JLA priority-setting partnership led by the eye research charity Fight for Sight (UK) has resulted in an extensive set of unanswered research questions prioritised by patients, carers and eye health professionals across 12 categories of eye conditions. Many of the major eye charities in the UK are encouraging grant applicants to address these research questions. It is also standard practice for patient representatives to sit on funding panels with a direct influence on how funds are allocated and, therefore, what research is undertaken. In addition, many medical charities have lay assessment panels to ensure that the voice of people living with a condition becomes central to discussion and influential in decision making when determining which research project grants should be recommended for funding.

In a major culture shift over the last decade, most funding agencies and ethical review panels now expect patients to be involved at all stages of a project as far as this is possible. This may include involvement in identification and prioritisation of the research area, study design, development of a grant proposal, undertaking and managing the research, analysis and interpretation, dissemination, implementation and monitoring and evaluation.[14] The requirement for investigators to document the extent of patient involvement across each of these domains within grant applications or submissions for ethical review provides a strong driver for research teams to increase PPI. It has also encouraged many research institutions to provide financial and personnel support for the setting up and running of PPI groups.

Discovering and measuring the patient experience of the disease and the impact of treatment

'I'm more than just a number': discovery through qualitative studies

The range of research questions in medical and healthcare research is broad. Many are concerned with hypothesis testing within the physical world, thus lending themselves to quantitative research designs. For example, research questions might examine the clinical and cost-effectiveness of treatments, the causes and patterns of disease or the detail of physiological and pathological disease processes. Examples in ophthalmology would be the following: 'In diabetic macular oedema, is drug A or drug B more effective in improving visual acuity at 3 months?' or 'In primary open-angle glaucoma, is drug A or a placebo more effective in lowering intraocular pressure?'.

However, there is also a range of research questions that address very different concerns that are crucially important to patient-focused research and practice. These might cover issues such as how patients experience, understand and interpret specific medical phenomena, such as the impacts of a diagnosis

Table 1 Ways in which the patient voice may be heard in healthcare (selected)

	Individual level	Collective and system level
Patient setting of the research agenda	Individual suggestions to a health professional or researcher	PPI, including: James Lind Alliance priority setting partnership Patient representatives on grant-awarding committees Awarding of grants dependent on PPI Patients as members of the research team
Patient experience of their disease and the impact of treatment	Dialogue with a health professional, for example, clinic visit PROMs	Qualitative studies Core outcome sets (COS) PROMs
Patient identification of their priorities for treatment	Dialogue with a health professional, for example, clinic visit PROMs	COS
Patient-to-patient peer support	Dialogue with another patient, for example, through PSS Online patient discussion forums	PSS-produced information resources PSS surveys of their members Research collating data from online forums
Patient assessment of quality of care	Individual feedback to a health professional or a peer	Patient satisfaction surveys PREMs Online resources (eg, reviews of services/individual clinicians)
Patient setting of health policy agenda	Individual requests to a health professional or policy maker	Patient representation at bodies such as HTA/NICE Campaigning by PSS

COS, core outcome sets; HTA, health technology assessment; NICE, National Institute of Health and Care Excellence; PPI, patient and public involvement; PSS, patient support society; PREMs, Patient-Reported Experience Measures; PROMs, patient-reported outcome measures.

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or of receiving treatment; the nature of patients' perceptions, attitudes and resulting behaviour in relation to their health and healthcare, such as adherence to treatment or clinic attendance; whether and how patients and carers adapt to life with chronic illness and what might facilitate this or what patients' and carers' priorities for treatment and recovery are and why. These are all examples of questions that require a different focus on the social and subjective human world. They are distinct from quantitative hypothesis testing and require more explorative qualitative research approaches to generate robust and meaningful insights.

Qualitative research methodologies have been developed over the last 50–60 years and are increasingly being used alongside quantitative health research to give complementary insights and understanding.[15] Some qualitative methodologies were originally conceptualised and developed in sociology, philosophy and anthropology and then applied to health research, while others have been adapted to fit the specific requirements of pragmatic applied health research.[16 17] Data collection and analysis will be influenced by the specific approach and research question.

Qualitative data collection techniques include in-depth interviewing, observations, focus group discussions and also visual forms of data collection, such as photo elicitation. In-depth interviews are common in health research and require in-depth participant-focused discussion between a researcher and a study participant. Qualitative observations may be undertaken by a researcher in natural settings, for example, as a non-participant observer of clinical practice, such as that taking place in an operating theatre, on a ward or during a patient consultation. As a consequence, qualitative data can be spoken, textual (transcripts and field notes from observations) or even visual (eg, photo elicitation or drawings). Analytical methods, which are specific to the research question, approach and data, produce findings that are represented by textual accounts and description, rather than quantitative measures reached by statistical means.[18]

In a manner similar to quantitative systematic reviews, there are techniques for the synthesis of research findings from different qualitative studies. Often, these aim to produce new insights that go above and beyond the original studies, rather than to be solely aggregative in nature,[19] as might be the case in a quantitative meta-analysis of trial data.

Quality criteria have been proposed for qualitative health research, and there is a long history of reflection on methodological rigour. However, due to the nature of research questions and methodological approach, it is inappropriate to judge qualitative studies according to traditional notions of research rigour as applied in quantitative health research.[20] Judgements regarding internal and external validity will depend on the research question and approach.

Qualitative research has the potential to significantly advance our understanding of the experience and motivations of patients with eye disease and to provide an alternate lens to view clinical practice. Examples of qualitative research are available in glaucoma and age-related macular degeneration (AMD). In the field of glaucoma, qualitative research has described patients' views on the experience of living with glaucoma,[21 22] what glaucomatous visual field loss 'looks like' from the patient viewpoint,[23] the experience of having a visual field test,[24] reasons for late presentation of the disease,[25] issues around follow-up, barriers to treatment, adherence[26] and opinions regarding personal health records.[27] Qualitative research is a valuable tool in exploring issues among vulnerable groups: Cross and colleagues have conducted a series of qualitative studies in the African-Caribbean population in the UK identifying themes that contribute to the high rates of blinding glaucoma in this population[28] and identifying routes to better patient–health professional engagement.[29] The impact of such studies is, in large part, due to their unfiltered reporting of the patient voice. For example, when Cross *et al*[28] asked African-Caribbean participants about access to primary eye care services in the context of glaucoma awareness, responses included: 'I believe I have to go to the doctor for the doctor to send me to see the eye optician', 'The thing is, I can see perfectly, I don't see a problem, but they find the problem', 'It scares me this glaucoma…if it happens, will I go blind, you know?'. In answer to the question 'Do you discuss this with the optician?', the challenging response is 'There's no opportunity. He seems more interested in selling me glasses'.

In AMD, studies have mainly focused on the experience of living with the disease,[30] the experience of receiving intravitreal treatment and treatment choice.[31] An example of a qualitative synthesis is the systematic review by Bennion *et al*[32] in 2012, which investigated the experience of patients living with AMD. This illustrated clear themes around the needs and perspectives of patients with AMD, including issues of stigma, social engagement and psychological impact.

In contrast to these two major blinding diseases, there are many areas of ophthalmology, such as uveitis, for which there has been very little qualitative research undertaken. The main exceptions are where investigators have been seeking to develop novel quality-of-life (QoL) measures for use in specific populations. Indeed, qualitative approaches are commonly used in the design of the content of quality of life measures to ensure that they have content validity from a patient perspective.[33–35] In the process of developing a novel QoL measure for the assessment of patients with birdshot chorioretinopathy (BCR), Barry *et al* conducted face-to-face interviews with a small group comprising two patients with BCR and one uveitis expert in order to generate 'items' for further evaluation in a series of questionnaires trialled in significantly larger groups. Similarly, when Angeles-Han *et al* wished to develop a novel QoL measure for use in children with juvenile-idiopathic-arthritis-associated uveitis, they conducted a series of interviews with professionals and children with various levels of visual impairment in order to generate their item bank. While studies such as these use qualitative data collection methods, they are expressly focused on developing quantitative measures of patient experience and QoL. In doing so, they may not necessarily realise the full potential value of qualitative approaches in affording the opportunity to understand patient experience and perspectives of disease and treatment in depth. However, in examples such as this, qualitative techniques contribute to quantitative research studies by ensuring that the latter assesses things that are of relevance to patients. In a similar manner, qualitative research designs are increasingly being integrated with quantitative trial research for a variety of purposes[36 37] and via mixed-method systematic reviews.[38] Quantitative and qualitative research are often conducted in parallel or in tandem to provide more rounded research insights into contemporary clinical questions.[39]

Qualitative research provides a unique way to hear the patient voice and the methodologies to help individuals and organisations learn from it. Qualitative research is undervalued and underused across most branches of ophthalmology but has enormous potential in advancing our understanding and improving the care for patients with eye disease.[21–32]

'It's about whether I can drive': prioritising outcomes that matter to patients through core outcome sets

There is a major problem in clinical research of lost opportunity, unused data and redundancy. This 'research waste' leads to the loss

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of 'billions of dollars of investment' and countless hours of patient and researcher time.[40] One major contributor to this 'waste' is the lack of standardisation between trials, which prevents comparison and aggregation of data. A major culprit is the use of numerous different endpoints to measure 'success'. This issue affects many branches of medicine. Within psychiatric medicine, a review of 2000 studies in schizophrenia found 640 different instruments used to measure outcome, 369 of which had only been used in one study.[41] In ophthalmology, a review of registered trials in uveitis found very high heterogeneity in the primary outcome measures used. Of 104 eligible trials, outcome measures were noted to arise from 14 different domains (such as visual acuity, vitreous haze or macular oedema, etc), but even among those trials measuring the same variable, there was considerable variation in how the variable was measured and what level or change was regarded as success.[42]

This heterogeneity of outcome makes it difficult to compare trials and often makes meta-analysis impossible. One solution would be to have a standard 'trial template' for each condition that prescribed key aspects of the study, including outcome measures (including method of measurement, timepoints and success threshold). While there would be many advantages, this would not allow the flexibility that, even within one disease, the range of potential interventions, clinical scenarios or patient cohorts require. A more practical solution is to agree on a set of relevant outcomes that will be collected in all trials for that condition, regardless of what the primary outcome is. These are known as core outcome sets (COS).[43] COS are not restrictive, since other data can be collected, but rather ensure that certain key outcomes are always collected in a standardised way. This may profoundly enhance evidence synthesis by (1) reducing heterogeneity (outcome measures are common to all relevant studies), (2) reducing outcome-reporting bias (there is a commitment to reporting the whole COS, and any omission is obvious) and (3) improving the statistical power of any meta-analysis (potentially all studies can be included).

Importantly, the emerging field of COS has provided an important new platform for the patient voice. Indeed, the patient voice within COS development has the power to strongly influence the whole process of drug evaluation and to ensure that trial endpoints include outcome measures that matter to patients. Organisations that promote COS development, such as COMET (Core Outcome Measures in Effectiveness Trials), have emphasised the need for COS to address the needs and priorities of all stakeholders. Rather than being determined solely by clinical experts, there is an expectation that patients, carers, trial experts and policy-makers all contribute to the process. The value of COS has also been endorsed by Cochrane, the GRADE (Grading of Recommendations, Assessment, Development and Evaluations) Working Group and the WHO.

COS development is challenging, and the methodology is evolving. Consensus development techniques such as a Delphi approach should be used to avoid undue influence from single individuals or groups in COS development. This approach may highlight discordance between the outcomes emphasised by different stakeholders, such as between clinicians and patients. There is, as yet, no 'gold standard' in terms of how to decide on the level of influence of the different groups and how this should be exercised throughout the process. This challenge is being addressed by studies investigating the influence of feedback and how patients' perceptions are altered when they are aware of the clinicians preferred COS.[44] One of the great advantages of the COS approach is its non-exclusivity, which means the final COS can include outcome measures that are important to individual groups and are not limited to only those that are prioritised by all groups.

In ophthalmology, relatively few COS have been published so far, but more are under development. For some conditions such as cataract, a single COS covering the whole condition has been proposed, whereas for others (notably uveitis), several different COS are envisaged to meet the specific requirements of the heterogeneity of the condition and differences in populations (eg, paediatric vs adult). Although some of these COS appear to be entirely based on clinical expert input (such as the COS for glaucoma effectiveness trials published by Ismail *et al*),[45] most recent COS include patients and other stakeholders; indeed, the number and type of contributors are explicit fields within the COMET registration to ensure that the degree of patient, carer and other stakeholder input is clearly evident (table 2).

'It's what I say that counts': measuring through patient-recorded outcome measures

Patient-reported outcome measures (PROMs) provide a way of recording patients' own measure of their health at any given time without clinician or researcher influence. The use of PROMs in ophthalmic research has been reviewed recently,[46] but it is worth highlighting here the extent to which PROMs may succeed or fail to communicate the patient voice.

Who decides whether a treatment is successful? In most clinical trials, success is defined by criteria set by clinicians or trialists. While such criteria may appear directly relevant to the patient (death being the most extreme example), many are surrogate measures of the disease process (such as intraocular pressure) or reflect a complication of the disease (for example, changes in central macular thickness on an optical coherence tomographic (OCT) scan). It needs to be recognised that many of these latter types of measures do not reliably correlate with the patient experience of their disease and may, therefore, not be an endpoint that they consider most relevant. Similarly, in routine clinical practice, overdependence on these measures may compromise patient care. An eye professional may have made his/her assessment of the patient's condition before entering the room. A patient with AMD who has stable visual acuity and no fluid on their OCT scan may be greeted with 'You're doing well aren't you?', potentially closing down their opportunity to communicate their worsening visual function and discuss their social isolation and associated depression.

PROMs are increasingly used both in clinical trials and routine clinical practice, potentially providing a systematic patient perspective on the impact of disease and its treatment. PROMs provide an opportunity to measure outcomes that resonate with patients. PROMs may provide assessments of health-related QoL or symptom burden, and some may also enable estimates of the health economic impact of disease/intervention. They are usually a secondary outcome measure in trials of effectiveness, in which they can inform whether an observed benefit in the primary outcome is complemented (or at least not negated) by the patient perception of symptoms or QoL; they can also be used as the primary outcome in research studies, although this is uncommon in ophthalmology.[47]

Within ophthalmology, PROMs have been used in a number of major trials across a range of conditions. The most common vision-related PROM used in clinical trials is the National Eye Institute Visual Function Questionnaire 25 (NEI VFQ-25). It was used as a secondary endpoint in several of the major trials of ranibizumab in AMD, such as ANCHOR[48] (Anti-VEGF (anti-vascular endothelial growth factor) antibody for the Treatment of Predominantly Classic Choroidal Neovascularisation in AMD) and MARINA[49] (Minimally Classic/Occult Trial of the Anti-VEGF

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Table 2 COS for ophthalmic disease registered with COMET (http://comet-initiative.org)

Disease area	Title of COS	Registered first author	Listed contributors	Date of publication
Cataract				
Cataract	ICHOM cataracts data collection reference guide	Lundstrom, M	Clinical experts, patient/support group representatives and methodologists	2014[84]
Retinal disease				
Age-related macular degeneration	ICHOM Macular Degeneration Data Collection Reference Guide	Gillies, M	Clinical experts, patient/support group representatives and methodologists	2015[85]
Age-related macular degeneration	Development of core outcome measures for age-related macular degeneration interventions	Azuara-Blanco, A	Consumers (patients) and clinical experts	In process
Diabetic retinopathy	Development of core outcome measures for diabetic retinopathy interventions	Azuara-Blanco, A	Consumers (patients) and clinical experts	In process
Inflammatory eye disease				
Uveitis	Defining a Core Outcome Set for Efficacy Trials in Adult Patients with Posterior Segment-Involving Uveitis	Denniston, A	Consumers (patients), clinical experts, governmental agencies, consumers (caregivers) and policy-makers	In process
Uveitis	Proposed outcome measures for prospective clinical trials in juvenile idiopathic arthritis-associated uveitis: a consensus effort from the multinational interdisciplinary working group for uveitis in childhood	Heiligenhaus, A	Clinical experts	2012[86]
Thyroid eye disease	Development of criteria for evaluating clinical response in thyroid eye disease using a modified Delphi technique	Douglas, R	Clinical experts	2009[87]
Glaucoma				
Glaucoma	Consensus on Outcome Measures for Glaucoma Effectiveness Trials: Results From a Delphi and Nominal Group Technique Approaches	Ismail, R	Clinical experts and researchers	2016[45]
Neuro-ophthalmic disease				
Amblyopia, strabismus and ocular motility	Development of a core outcome set for clinical research and practice in amblyopia, strabismus and ocular motility disorders	Al-Jabri, S	Consumers (patients), journal editors, clinical experts, researchers, consumers (caregivers), methodologists, policy-makers, service providers and statisticians	In process
Stroke	Impact of Visual Impairment after Stroke	Rowe, F	Consumers (patients), clinical experts, researchers, academic research representatives, consumers (caregivers), policy-makers, service providers, statisticians, patient/support group representatives, members of a clinical trial network, study investigators and service users	In process

COS, core outcome sets; COMET, Core Outcome Measures in Effectiveness Trials; ICHOM, International Consortium for Health Outcomes Measurement.

Antibody Ranibizumab in the Treatment of Neovascular AMD), both of which showed an improvement in NEI VFQ-25 in line with the measured improvement in visual acuity. The value of the tool is evident in publications such as Bressler et al's combined analysis of the two trials, which showed that treatment with ranibizumab led to an improvement in QoL (as measured by NEI VFQ-25), whether the treated eye was the better or worse seeing eye (although the benefit was smaller when it was the worse seeing eye).[50] They also showed that greater gain in measured visual acuity (VA) was associated with higher NEI VFQ-25 gain in terms of overall score and the subscales of near activities, distance activities and vision-specific dependency.[51] It should be noted that a number of investigators opt to use PROMs that are more specific to their condition; for example, some AMD studies, such as IVAN[52] (Inhibition of VEGF in Age-related Choroidal Neovascularisation Trial), used the macular-specific QoL measure MacDQoL (impact of Macular Disease on Quality of Life).[53]

In glaucoma, the first major trial to use a visual function and QoL PROM was the CIGTS[54] (Collaborative Initial Glaucoma Treatment Study), which used the original 51-item version of the NEI VFQ, and an older tool, the Visual Activities Questionnaire (VAQ). In this study, most patients with glaucoma reported high levels of visual functioning (overall score and in most subscales), but that increasing visual field loss (as measured by perimetry) was associated with a significant decrease in the overall score and in the peripheral-vision subscale. In addition, it was noted that the patients' perception of their visual function was most commonly associated with visual field in the 'better' eye and visual acuity in the 'worse' eye.[54] In later studies, such as the EMGT[55] (Early Manifest Glaucoma Trial) and EAGLE[47] (Effectiveness in Angle Closure of Lens Extraction), the NEI VFQ-25 version has been used, consistently showing a decline in visual-function-related QoL with advancing field loss.[56] Glaucoma is a good example of a condition in which the experience of the disease for the patient may be very different from many other sight-threatening conditions due to the predominance of field loss rather than early loss of visual acuity. For this reason, disease-specific tools such as the Glau QoL 36 may commonly be used alongside or instead of more generic tools such as the NEI VFQ-25. The review by Quaranta *et al*[57] provides a recent review of the range of QoL PROMS used in glaucoma and their findings.

Although numerous vision-related PROMs are available, the NEI VFQ-25 was developed for broad applicability and has been shown to be useful across a range of sight-impacting conditions. It has been used as a secondary endpoint in therapeutic trials for many conditions (see Table 3), including diabetic macular oedema (eg, RESTORE),[58] macular hole surgery (FILMS),[59] retinal vein occlusions (eg, BRAVO and CRUISE)[60] and posterior segment uveitis (eg, MUST).[61–63] It remains the most commonly used visual function PROM, although it may sometimes be delivered in parallel to a more 'targeted' disease-specific measure.

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Table 3 Summary of resources, abbreviations and acronyms

Acronym	Full name	Description
ANCHOR	Anti-VEGF Antibody for the Treatment of Predominantly Classic Choroidal Neovascularization in Age-Related Macular Degeneration	Phase 3 clinical trial in age-related macular degeneration
BRAVO	Ranibizumab for the treatment of Macular Oedema Following Branch Retinal Vein Occlusion: Evaluation of Efficacy and Safety Trial	Phase 3 clinical trial in Branch Retinal Vein Occlusion (BRVO)
CIGTS	Collaborative Initial Glaucoma Treatment Study	Medical versus surgical therapy trial in open-angle glaucoma
COMET	Core Outcome Measures in Effectiveness Trials	An initiative to bring together people interested in the development and application of agreed standardised sets of outcomes, known as COS
COS	Core Outcome Sets	An agreed minimum set of outcomes or outcome measures to be collected in all clinical studies of a particular condition
COSMIN	Consensus-Based Standards for the Selection of Health Measurement Instruments	An initiative to improve the selection of health measurement instruments
CRUISE	Central Retinal Vein Occlusion Study: Evaluation of Efficacy and Safety Trial	Phase 3 clinical trial in Central Retinal Vein Occlusion (CRVO)
EAGLE	Effectiveness of Early Lens Extraction for the Treatment of Primary Angle-Closure Glaucoma	Surgical trial in primary angle-closure glaucoma
FILMS	Full-Thickness Macular Hole and Internal Limiting Membrane Peeling Study	Surgical trial in macular-hole repair
FVQ CYP	Functional Vision Questionnaire for Children and Young People	Questionnaire for the measurement of visual function in children and young people
HTA	Health technology assessment	The systematic evaluation of the properties, effects and/or impacts of a health technology
ICHOM	International Consortium for Health Outcomes Measurement	An initiative to try to measure and report patient outcomes in a standardised way
ISOQOL	International Society for Quality of Life Research	An initiative to advance the study of health-related quality of life and other patient-centred outcomes
IVAN	Inhibition of VEGF in Age-Related Choroidal Neovascularization	Randomised controlled trial in age-related macular degeneration
MacDQol	Macular Disease on Quality of Life	An individualised measure of the impact of macular disease on quality of life
MARINA	Minimally Classic/Occult Trial of the Anti-VEGF Antibody Ranibizumab in the Treatment of Neovascular AMD	Phase 3 clinical trial in age-related macular degeneration
MUST	Multicentre Uveitis Steroid Treatment	Phase 4 clinical trial in uveitis
NEI VFQ-25	National Eye Institute Visual Functioning Questionnaire 25	Questionnaire for the measurement of visual function in adults
PREM	Patient-reported experience measure	A measurement instrument by which patients assess their experience of one or more aspects of their healthcare
PROM	Patient-reported outcome measure	A measurement instrument by which patients assess their own health status, which may include symptoms, functionality and other aspects of physical, mental and social health
PROQOLID	Patient-Reported Outcome Quality of Life Instruments Database	A database of patient-centred clinical outcome assessments
PSS	Patient Support Society	An organisation that exists to support patients, usually outside the healthcare institutions
RESTORE	Ranibizumab Monotherapy or Combined with Laser versus Laser Monotherapy for Diabetic Macular Edema	Phase 3 trial in diabetic macular oedema
VAQ	Visual Activities Questionnaire	Questionnaire of visual function in adults
VQoL_CYP	Vision-Related Quality-of-Life Instrument for Children and Young People	Questionnaire for the measurement of vision-related quality of life in children and young people

It should be noted that most ophthalmic PROMS are geared to an adult population and may fail to capture the experience of children with ophthalmic disease. Specific age-appropriate tools such as the VQoL_CYP (Vision-Related Quality-of-Life Instrument for Children and Young People)[64] and the FVQ_CYP (Functional Vision Questionnaire for Children and Young People)[65] have enabled important insights into the experience of children living with visual impairment. An important aspect of this is the recent work by Rahi and colleagues, which demonstrated that visually impaired children and their parents differ significantly in their assessment of the impact of living with visual impairment. On average, parents scored their child's vision-related QoL and functional vision worse than the child's own assessment, but with a wide range of disagreement.[66]

Almost all the PROMs considered so far, including the NEI VFQ-25, are focused on visual function and vision-related QoL, and thus limit the patient views to these domains. A number of other PROMs provide an opportunity for patients to express a more holistic assessment of their health state and function. These more generic instruments include both wider assessments of health (such as the Short-Form 36)[67] and assessments of 'utility', which provide a way of scoring health states across different conditions using units such as 'Quality-Adjusted Life Years (QALYs)'. The most commonly used utility measure in ophthalmic trials is the EQ5D,[68] a simple questionnaire that includes four questions regarding health state and then a self-rating visual analogue scale between 0 and 100. The inclusion of a utility measure such as the EQ5D provides a way to estimate the incremental effectiveness of an intervention in terms of QALYs, which can then provide a more complete assessment of cost-effectiveness. This may be particularly important for health technology assessments and policy making when comparing multiple interventions for which the clinical effectiveness is similar. Utility measures are very rarely used as a primary outcome measure—EAGLE is an exception in this regard—but their use in major pharmacological studies as a secondary endpoint is increasing.

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In routine clinical practice, PROMs have been adopted in some areas to provide patient feedback on the success of common procedures; in the UK, for example, PROM assessment is standard before and after surgery for inguinal hernia, varicose veins and knee and hip replacement.[69 70] Although commonly administered in paper questionnaire form, PROMs are amenable to electronic administration, which can significantly reduce the burden on data collection for the clinical and research teams.[71–73] Since PROMs are in a standardised questionnaire format, they can be subject to quantitative analysis and relatively easily summarised statistically to show the direction and size of change in the parameter being measured. It would seem then that PROMs are an ideal vehicle for the 'patient voice' to be heard loud and clear in both research and clinical domains.

There are, however, significant issues with our current usage of PROMs, both generally and specifically within ophthalmology. It is one thing to gather information; it is another to act on it. There is a concern that many trials include the collection of PROM data but fail to publish the results.[74 75] This represents a huge loss in terms of patient and researcher hours completing questionnaires and constitutes serious 'research waste'.[40] In addition, patients may believe that they are communicating with the clinical team by filling in such a questionnaire and may indeed express serious concerns or anxieties within it, which are not otherwise verbalised to the clinical team. The handling of such 'danger signals' within PROM collection, known as a 'PRO Alert', is the subject of ongoing research.[76] Other challenges include the 'crowded marketplace', where selection of a suitable PROM may be confounded by the many different PROMs of variable quality purporting to achieve broadly similar aims. While there is certainly a place for both generic and specific PROMs, a lack of consistency between trials reduces the opportunity to compare between studies. The choice of PROM may be assisted by resources such as the PROQOLID (Patient-Reported Outcome Quality-of-Life Instruments Database),[77] an online database that provides key information about each PROM. Furthermore, PROMs can be evaluated using the COSMIN (Consensus-Based Standards for the Selection of Health Measurement Instruments),[78] critical appraisal tool, and a minimum measurement standard when using a PROM is addressed by the International Society for Quality of Life Research.[79] It is also important that patients are involved with the process of PROM selection to ensure both acceptability and appropriateness (see table 3 for summary of resources, abbreviations and acronyms).

'And while we're talking…': other areas informed by the patient voice

The major focus of this review has been on hearing the patient voice as it pertains to patients' experience of disease, the impact of treatment and the patient priorities for outcome. As highlighted in table 1, there are numerous ways in which the patient voice may be heard at the individual and collective levels which will impact healthcare. Peer-to-peer support has become much easier in the digital age, and its reach and immediacy has exploded with the advent of discussion forums and social media. This also means that there is no longer any 'control' of such discussions by the medical establishment, although some routes, such as patient support society forums, may be subject to 'moderating' by a patient expert. The collective wisdom and experience of patients may also be gathered formally by patient support societies through questionnaires that survey their members on a wide range of issues. Indeed, in rare conditions, this may be the only way to access a large number of people with that particular disease. For example, in uveitis, this approach has been carried out successfully in Behcet's disease, punctate inner choroidopathy[80] and birdshot chorioretinopathy.[81]

The patient voice can also directly influence health policy and affect the provision of treatment at the national level. Many regulatory bodies and health technology assessment panels invite comments from stakeholders, including patient groups. It is hard to quantify fully the extent of patient influence on these policy decisions, but it would appear that the mobilisation of vocal patient societies and other organisations can cause a change in policy. This is commonly around the provision of expensive drugs and which groups are eligible. An interesting example from the UK was during the advent of ranibizumab for AMD, where the advisory body the National Institute of Health and Care Excellence published draft guidance that would have limited anti-VEGF therapy to treatment of the 'better eye' only. After representation from patient groups, including the Macular Society and the Royal National Institute for the Blind, in addition to strong support from clinical experts through organisations such as the Royal College of Ophthalmologists, the final report was revised to enable treatment of either eye, stating that '[The Committee] noted the concerns raised by consultees and understood that most consultees felt that it would be unacceptable, and clinically inappropriate, not to treat the first eye that comes to clinical attention'.[82] Lay members (and this could include patients and carers) also play an integral and important role in the training and education of doctors and sit on many medical committees. For example, in the UK, this includes the General Medical Council, British Medical Association, medical royal colleges and local education and training boards, as well as clinical commissioning groups and research ethics committees.

Although the 'campaign reach' and influence of large organisations are unsurprising, the impact of individual patients should not be underestimated. The stories of individual patients may catch media attention and lead to public campaigns that become very difficult for governments and their agencies to resist. This clearly presents problems in a resource-limited situation, since the unseen competition between funding for different conditions effectively pitches the voice of one patient (or patient group) against another's. There is a danger here that allocation may be overly influenced by who shouts the loudest.

One of the most challenging areas for clinicians and health providers is where the patient voice is critical. Patient feedback is one of the most valuable resources to inspire and direct improvement in healthcare, but it can also be perceived by some as threatening to both the individual and the institution. Organisations may proactively seek this feedback, usually with a quantitative component, so that they can measure their rating over time. Various tools can be used, including validated patient-reported experience measures (PREMs), and within ophthalmology, the use of patient-reported outcome and experience measures (POEMs), which combine elements of PROMs and PREMs into a single brief questionnaire, has been trialled.[83] In contrast to the feedback solicited by the health providers, much evaluation now is spontaneous unsolicited expressions of complaint, compliment or comment. Opportunities include the official social media sites of the organisation (providing true feedback and allowing the organisation to respond) and completely independent sites (providing information for other consumers but without necessarily feeding back to the organisation).

'Why aren't you listening?': expectations and limitations

Although the emphasis of this review is on valuing the patient voice and increasing its influence in healthcare, there are challenges and constraints. The patient voice is not the only voice. As

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we enable the patient voice to be heard in ophthalmic research and clinical care, we need to be ready for potential disagreement and disappointment. Dialogue between the patient, research team, caregivers and, indeed, society should recognise these differences in perspective between individuals and stakeholder groups.

Patients are, indeed, better placed than anybody else to speak about their disease and the impact of treatment as it pertains to them. Their experience, knowledge and decision making is, however, specific. They have experienced one particular journey through that disease and a selected range of treatments; their knowledge of the disease may be incomplete, and their decision making will be strongly influenced by their own experiences and outcomes. The approaches we have discussed in this review, including qualitative research, PROMs, COS and experience measures, may provide a way to value the individual view and enable a more macroscopic perspective on common themes that are common to one or more patient groups. At a societal level, the influence of the collective patient voice will be moderated by context, notably the limitation of resources and the competing needs of different groups of patients.

CONCLUSION: REALIGNING OPHTHALMIC CARE THROUGH HEARING THE PATIENT VOICE

This narrative review has considered the central importance of the 'patient voice'. We have considered how the patient perspective can be collected in both research and clinical contexts and how this can inform our systems of care and direction of research within ophthalmology. Traditional objective clinical measures such as visual acuity are important but are one-dimensional and fail to provide an adequate assessment of their condition. Learning to hear the 'patient voice' in clinical practice and research enables a more complete understanding of what patients are actually experiencing through their disease and what their priorities are for treatment. We need to move from the patient being the 'object' of patient-centred care to being the 'subject'. Only then will the care and research agenda be realigned according to patient priorities and become truly patient centred. After all, in the final analysis of healthcare, 'patients are the measure of all things'.

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