Defining a Minimum Set of Standardized Patient-centered Outcome Measures for Macular Degeneration

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• PURPOSE: To define a minimum set of outcome measures for tracking, comparing, and improving macular degeneration care.

• DESIGN: Recommendations from a working group of international experts in macular degeneration outcomes registry development and patient advocates, facilitated by the International Consortium for Health Outcomes Measurement (ICHOM).

• METHODS: Modified Delphi technique, supported by structured teleconferences, followed by online surveys to drive consensus decisions. Potential outcomes were identified through literature review of outcomes collected in existing registries and reported in major clinical trials. Outcomes were refined by the working group and selected based on impact on patients, relationship to good clinical care, and feasibility of measurement in routine clinical practice.

• RESULTS: Standardized measurement of the following outcomes is recommended: visual functioning and quality of life (distance visual acuity, mobility and independence, emotional well-being, reading and accessing information); number of treatments; complications of treatment; and disease control. Proposed data collection sources include administrative data, clinical data during routine clinical visits, and patient-reported sources annually. Recording the following clinical characteristics is recommended to enable risk adjustment: age; sex; ethnicity; smoking status; baseline visual acuity in both eyes; type of macular degeneration; presence of geographic atrophy, subretinal fibrosis, or pigment epithelial detachment; previous macular degeneration treatment; ocular comorbidities.

• CONCLUSIONS: The recommended minimum outcomes and pragmatic reporting standards should enable standardized, meaningful assessments and comparisons of macular degeneration treatment outcomes. Adoption could accelerate global improvements in standardized data gathering and reporting of patient-centered outcomes. This can facilitate informed decisions by patients and health care providers, plus allow long-term monitoring of aggregate data, ultimately improving understanding of disease progression and treatment responses. (Am J Ophthalmol 2016;168:1–12. Published by Elsevier Inc. This is an open access article under the CC BY-NC-ND license (http://creativecommons.org/licenses/by-nc-nd/4.0/).)

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MACULAR DEGENERATION IS A LEADING CAUSE OF irreversible vision loss, accounting for over 15% of blindness in high-income countries, with the burden of this disease expected to increase with aging populations.[1] Age-related macular degeneration (AMD) is the most prominent macular degeneration in populations of European descent. Even in Asian populations, who are known to have a lower risk of AMD than people of European descent, AMD is one of the leading causes of blindness.[2] Although non-neovascular AMD is much more common, untreated neovascular AMD used to frequently be responsible for severe vision loss. Less

common types of neovascular macular degeneration include those secondary to myopia, trauma, inflammation, macular telangiectasia, and idiopathic causes, all of which, when associated with choroidal neovascularization (CNV), are broadly treated with a similar approach as that for neovascular AMD. Intravitreal anti–vascular endothelial growth factor (anti-VEGF) therapy has become the established treatment of neovascular AMD over the last decade, supported by evidence from pivotal randomized clinical trials.[3,4]

Interventional clinical trials are designed to maximize the likelihood of demonstrating a treatment effect and therefore often use a study population that may be significantly different from the general population of patients with the disease in question.[5] For these and other reasons, including less frequent treatment and less regular follow-up than is inherent in a clinical trial, outcomes achieved in the pivotal trials are not always replicated in routine clinical practice.[6,7] Interventional clinical trials also suffer from relatively small numbers of patients who are treated and short duration of follow-up, with only 3 major prospective studies reporting data beyond 2 years of treatment.[8–10] This is particularly problematic with macular degeneration being a lifelong disease, for which the long-term outcomes, especially with treatment, are less well understood.

Various regimens have been developed to address the vast burden of treatment for neovascular macular degeneration. Monthly dosing, which was initially recommended,[11] has been largely supplanted by treat-and-extend[12] or variable as-required (pro re nata) dosing.[13] Decisions regarding initiating treatment and retreatment vary internationally and according to reimbursement schemes, but are generally governed by changes in visual acuity and CNV activity as determined by the detection of intraretinal and subretinal fluid using optical coherence tomography (OCT).[14] There is also increasing choice in anti-VEGF agents (ranibizumab, bevacizumab, aflibercept) and numerous potential treatments for non-neovascular AMD, which are currently being evaluated in clinical trials.[15]

The limitations of clinical trials, variation in treatment approaches and therapies, and ever-growing financial burden of providing long-term treatment despite uncertain long-term gains indicate that the need for systematic measurement of the outcomes of macular degeneration treatment in routine clinical practice is greater than ever. However, only a few care providers around the world routinely record outcome data for the treatment they give to patients for macular degeneration outside of clinical trials. There are, however, emerging "registries"—national or multinational data repositories that assimilate clinical outcome data from large numbers of individual practices. These include the significant projects originating in Sweden (The Swedish Macula Register),[16] Australia (The Fight Retinal Blindness! Project),[17] the United Kingdom (The UK National Ophthalmology Database),[18] and the Czech Republic (The Amadeus Project).[19] The largest is the Luminous Project, which has recently completed enrollment of 30 000 patients across 5 continents (including up to 10% of patients undergoing intravitreal anti-VEGF treatment for diabetic macular edema and retinal vein occlusion); however, this only includes patient receiving ranibizumab.[20] In the United States, the American Academy of Ophthalmology Intelligent Research in Sight (IRIS) project, which was launched in 2014, is the first comprehensive eye disease clinical registry, with macular degeneration outcome measures in development.

Although existing efforts to measure outcomes of macular degeneration treatment from routine clinical care are a good start, there is no agreed standard approach, and therefore significant variation exists in what outcomes are recorded as well as how they are measured and reported. An overview of the frequency at which main outcomes are currently being measured in existing registries and major clinical trials is provided in Figure 1. The figure shows that visual acuity and complications of treatment are commonly included. However, long-term disease control and anatomic outcomes are tracked in fewer than half of the registries and trials. The absence of an agreed standard approach limits the ability to perform direct comparisons of different services that could lead to improved outcomes. Most importantly, functional outcomes that are more meaningful to patients may be underrepresented compared to clinical or anatomic outcomes, which are easier to measure but may bear less direct relevance to patients.

Standardized and patient-centered outcome measurement is therefore crucial in order to direct improvements by those providing treatment, promote dissemination of best practices, and ultimately drive competition around quality. Systematic studies of interventions for other diseases in routine practice have exposed significant variation in outcomes that appeared to be dependent on differences in institutional methods, physician preference, or health care systems.[21] Continued data collection and outcome reporting has subsequently led to clear improvements in these outcomes.[22,23] For these reasons, a Working Group came together, facilitated by the International Consortium for Health Outcomes Measurement (ICHOM, a nonprofit organization with the purpose to transform health care systems worldwide by measuring and reporting patient outcomes in a standardized way), to develop an international, standardized, core set of patient-centered outcomes measures, with common definitions for routine clinical data collection in macular degeneration care. It is hoped that the minimum set of outcomes that we propose will be used as a common platform for routine clinical data collection by those providing care for patients with macular degeneration.

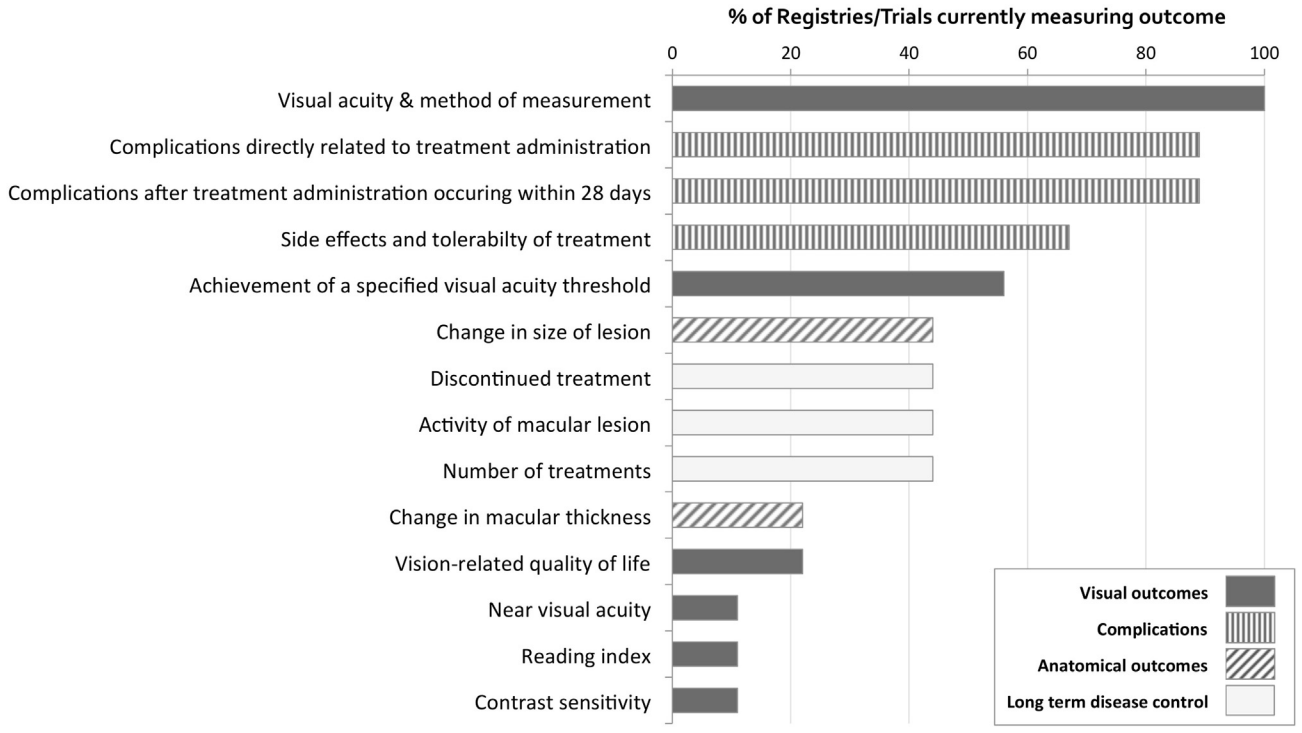

FIGURE 1. Frequency of main outcomes measured in existing registries and major clinical trials. The registries included are Amadeus Project, Fight Retinal Blindness! Project, Luminous Project, Swedish Macula Register, and United Kingdom National Ophthalmology Database. The trials included are ANCHOR, CATT, IVAN, and MARINA.

METHODS

THE ICHOM APPROACH TO DEFINING HEALTH OUTCOMES for conditions involves forming an international working group that follows a structured approach to compile the key outcomes and clinical characteristics that are recommended to be measured in routine clinical practice, called the "Standard Set." The work was supported by sponsorship from a number of charitable health care organizations, but has no financial support from any pharmaceutical or health care technology organizations.

• **INTERNATIONAL WORKING GROUP:** ICHOM convened a working group of 18 members, representing 10 different countries from 4 continents (Figure 2). The working group consisted of leading experts in the fields of macular degeneration outcomes and health care registry development, as well as patient advocates. The working group lead, Professor Mark Gillies, has previously led the development of the collaborative Fight Retinal Blindness! (FRB!) Project,[24] which is an efficient, web-based system to track outcomes of patients receiving treatment for neovascular AMD in clinical practice. A project leader from ICHOM (S.S.) managed the project, and an ICHOM Research Fellow (I.R.) supported the content development. The other ophthalmologists among the working group had experience in leading the retinal services in internationally recognized ophthalmic units and established clinical data registries in North America, Europe, and Asia. The patient representatives had direct experience of treatment for macular degeneration and were involved in national charitable organizations for macular disease, which gave them access to the insight of thousands of other patients with macular degeneration.

• **DEVELOPMENT OF STANDARD SET:** A modified Delphi technique was used to develop these recommendations, in which a sequence of rounds of discussion followed by an electronic survey were used to arrive at a consensus among the experts who had differing views and perspectives. This enabled the input from participants to be gathered without requiring them to work face-to-face to find consensus.

Six teleconferences were used to discuss outcomes and clinical characteristics for inclusion in the Standard Set. The first teleconference focused on the scope and outcome domains for the Standard Set, supported by a literature review of existing registries and major clinical trials to produce a long list of outcome measures that were being recorded at the time.

The long list of potential outcomes was refined through consensus discussions steered by the working group lead in

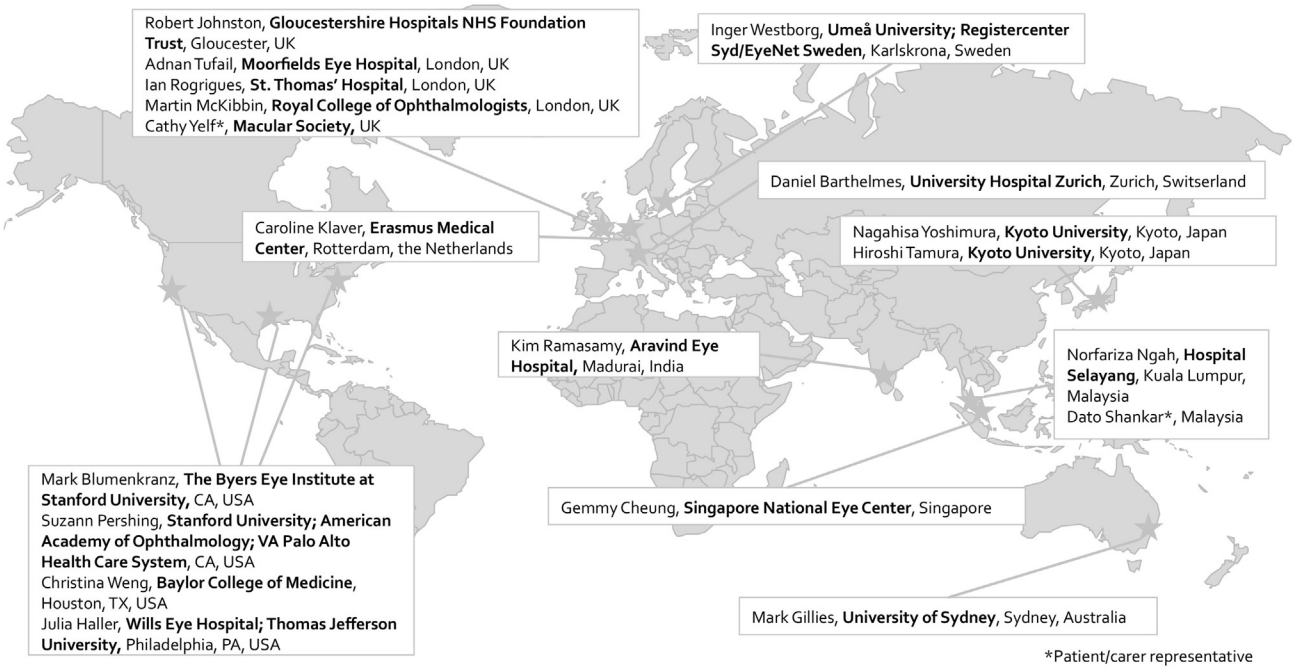

FIGURE 2. The International Consortium for Health Outcomes Measurement (ICHOM) macular degeneration working group.

order to prioritize outcomes that (1) had direct impact on patients; (2) were sensitive to good clinical care; and (3) were feasible to measure in routine clinical practice.

Following the teleconference, members submitted their feedback and final votes on the inclusion of each outcome through electronic surveys. A 75% majority of initial votes was used as the threshold for inclusion of the outcome, and below 50% was the threshold for rejecting outcomes. In cases where a particular outcome received votes for inclusion between 50% and 75%, the contentious point was revisited on the next call and survey. The second teleconference covered the measurement and definition of the selected outcomes.

A similar process was undertaken in teleconferences 3 and 4 to identify the clinical characteristics that are recommended to be measured in order to risk-stratify patients in subsequent analyses. The measurement and definition of the selected clinical characteristics was also agreed upon. Publication, adoption, and implementation strategies were discussed in teleconferences 5 and 6. The final Standard Set was refined and approved unanimously by all members of the working group.

RESULTS

A SUMMARY OF THE ICHOM MACULAR DEGENERATION Standard Set is shown in Tables 1–3, with additional details in the sections below. A data collection manual that further describes each measure, its definition, suggested reporting format, inclusion and exclusion criteria, and potential data sources is freely available on the ICHOM Website (http://www.ichom.org/medical-conditions/macular-degeneration/).

• **CONDITIONS AND TREATMENT APPROACHES COVERED:** The Standard Set was developed to include the broad disease categories of macular degeneration and their treatments that were deemed to be sufficiently different to warrant separation, as listed in Table 1. Other forms of non-neovascular macular degeneration were excluded owing to the large variation in the pathogenesis, prognosis, and treatment of such diseases, which would therefore require measurement of different clinical characteristics and outcomes.

• **OUTCOMES RECOMMENDED IN THE INTERNATIONAL CONSORTIUM FOR HEALTH OUTCOMES MEASUREMENT MACULAR DEGENERATION STANDARD SET:** A large number of potential outcomes were considered by the working group before agreement on the final minimum set of outcomes that are recommended for the affected eye, as detailed below. Further details of these potential outcomes, including the final voting decisions and summary of the working group discussions that led to a decision of inclusion or exclusion of individual outcomes, are included in Supplemental Table 1 (Supplemental Material available at AJO.com).

Visual Functioning and Vision-related Quality-of-Life Outcomes. Because improving or maintaining visual

TABLE 1. Conditions and Treatments Included in the International Consortium for Health Outcomes Measurement Macular Degeneration Standard Set

Conditions Included	Neovascular macular degeneration:
	Neovascular AMD
	Polypoidal choroidal vasculopathy
	Myopic neovascular macular degeneration
	Other forms of neovascular macular degeneration (includes post-traumatic, inflammatory, idiopathic, macular telangiectasia type 2)
	Non-neovascular AMD
Treatments Included	Intravitreal anti-VEGF treatment
	Intravitreal steroids
	Photodynamic therapy
	Thermal laser photocoagulation
	Retinal radiation therapy
	Transpupillary thermotherapy
	Retinal surgical treatment
	Other

AMD = age-related macular degeneration; VEGF = vascular endothelial growth factor.

function is the primary goal for most patients with macular degeneration, it is clearly an essential component of any outcome assessment. We recommend recording of distance visual acuity (best of uncorrected, corrected using glasses or contact lenses, or pinhole if required) in the affected eye at each clinical visit. Results should preferably be measured using a logarithm of the minimal angle of resolution (logMAR) or ETDRS chart, although other measurement systems may be used and subsequently converted to logMAR visual acuity.

When analyzing distance visual acuity outcomes of treated eyes, we recommend reporting the following data:

- Mean change in visual acuity from baseline
- Proportion of eyes gaining vision (≥5 logMAR letters)
- Proportion of eyes with stable vision (within 15 logMAR letters of baseline)
- Proportion of eyes with visual acuity of ≤0.3 logMAR (20/40 Snellen)
- Proportion of eyes with visual acuity of ≥1.0 logMAR (20/200 Snellen)

We chose to recommend measuring mean change in visual acuity after starting treatment because it has become the primary outcome of phase III clinical trials for neovascular AMD.[4,6] However, observational studies have found that, because of ceiling effects, this may skew results in favor of services that detect the disease and start treatment late, since eyes with worse vision have more to gain compared with eyes starting with good vision, which may not gain anything.[20] For this reason we recommend that the proportion of eyes with stable vision, good vision (≥20/40), and poor vision (≤20/200) should also be measured.

There is mounting evidence that increases in objectively measured distance visual acuity do not necessarily concur with improved visual functioning for patients.[25] Therefore, to fully measure the impact of macular degeneration treatment, it is important to also assess how the patient's vision-related quality of life has changed. The working group felt that measures of visual function other than distance visual acuity, such as near visual acuity and reading speed, were important outcomes. However, rather than requiring these to be captured as clinical data in a clinical setting, they recommended them to be measured collectively via subjective patient reporting from real life via a patient-reported outcome measure (PROM). The working group felt that use of PROM data would provide a superior reflection of the visual function of patients in the real world than clinical measures such as contrast sensitivity and that PROM data would be of use in directing treatment.

We evaluated a number of existing, validated PROMs that are potentially suitable for assessment of visual function affected by macular degeneration. These included the Daily Living Tasks Dependent on Vision (DLTV), Impact of Vision Impairment (IVI), Macular Disease Quality of Life (MacDQoL), Metamorphopsia Questionnaire, and the National Eye Institute Visual Functioning Questionnaire (NEI-VFQ), which have been recently reviewed by Khadka and associates.[26] The criteria that we used to evaluate all the relevant PROMs consisted of the following: (1) content of the PROM (based on domains of visual function that the working group felt were most important to patients with macular degeneration, including work/hobby continuation, face recognition/social participation, driving/transport, reading, self-care, general enjoyment of life, activities involving near vision, activities involving distance vision, bodily symptoms/functions); (2) quality of the PROM (psychometric properties, content development, reliability, and validity specifically for macular disease); and (3) practical aspects related to using the PROM (available languages, time taken to complete, costs involved with using in routine clinical care).

Based on our extensive evaluation and discussions, we recommend the use of the Impact of Vision Impairment (IVI) questionnaire[27] at baseline, prior to initiating treatment and annually while under follow-up. The importance of follow-up assessment is to quantify changes in response to treatments and to allow comparison between different care providers. Annual reassessment has been recommended to minimize data collection burden but enable meaningful changes to be detected in a reasonable period of time. The IVI has been validated specifically in patients with AMD and found to have appropriate content development and reliability, plus has undergone Rasch analysis.[28] It also scores highly on its psychometric properties, including unidimensionality within each domain and high measurement precision.

TABLE 2. Summary of Outcomes Recommended in the International Consortium for Health Outcomes Measurement Macular Degeneration Standard Set

	Measure	Details	Timing	Data Source
Visual functioning and vision-related quality of life	Distance visual acuity	Distance visual acuity (best of uncorrected, corrected, or pinhole) in the affected eye. Change in distance visual acuity should be calculated from baseline and previous visual acuity assessments.	• Each clinical visit	Clinical data
	Mobility and independence	Impact of Vision Impairment questionnaire	• Baseline (prior to treatment) • Annually (while on treatment)	Patient reported
	Emotional well-being	Impact of Vision Impairment questionnaire	• Baseline (prior to treatment) • Annually (while on treatment)	Patient reported
	Reading and accessing information	Impact of Vision Impairment questionnaire	• Baseline (prior to treatment) • Annually (while on treatment)	Patient reported
Disutility of care	Number of treatments	Documentation of individual treatments received for macular degeneration (Table 1)	• Each clinical visit	Clinical or administrative data
	Complications of treatment[a]	Endophthalmitis: Severe intraocular inflammation within 3 months of last intraocular treatment, due to infectious or noninfectious causes	• Each clinical visit	Clinical data
Disease control	Presence of fluid, edema, or hemorrhage[a]	Presence of intraretinal or subretinal fluid or hemorrhage that is attributable to activity of the neovascular lesion as determined by the treating ophthalmologist. This could be based on clinical examination or imaging.	• Each clinical visit	Clinical data

[a]Outcomes applicable to neovascular macular degeneration only.

In terms of content, the IVI consists of 28 items that cover an appropriate and broad range of questions that comprise 3 domains: "mobility and independence," "emotional well-being," and "reading and accessing information." The questions assess bodily symptoms and functions, visual tasks and activities (in particular, those requiring near vision), social participation, and emotional well-being, which were all felt to be true outcomes of high importance and relevance for patients with macular degeneration. We do recognize that other PROMs are widely used and can provide valuable information; however, our decision to recommend the IVI was to ensure that all these important patient needs can be captured.

In practical terms, the IVI is also free to use (for noncommercial purposes) and takes approximately 15 minutes to complete using paper, computer, or touch screen–adapted versions. A guide to scoring and analysis of the IVI is included in the ICHOM Macular Degeneration Standard Set Reference Guide (available at http://www.ichom.org/medical-conditions/macular-degeneration/). It

TABLE 3. Summary of Clinical Characteristics and Interventions Recommended in the International Consortium for Health Outcomes Measurement Macular Degeneration Standard Set

	Measure	Details	Timing	Data Source
Patient demographics	Age	Age in years, calculated from birth date at commencement of therapy	• At baseline	Clinical or patient reported
	Sex	Sex at birth	• At baseline	Clinical or patient reported
	Ethnicity	Asian, Black, Hispanic, white, mixed, other	• At baseline	Patient reported
	Smoking status	Smoking status (of cigarettes, cigars, or tobacco)	• At baseline	Patient reported
Baseline functional status	Baseline visual acuity	Distance visual acuity (best of uncorrected, corrected using glasses or contact lenses, or pinhole) in the affected eye	• At baseline	Clinical data
	Baseline visual acuity in the fellow eye	Distance visual acuity (best of uncorrected, corrected using glasses or contact lenses, or pinhole) in the fellow eye	• At baseline	Clinical data
Clinical status	Type of macular degeneration	Type of macular degeneration as defined in Table 1	• At baseline • Prompt at each clinical visit to check for change	Clinical data
	Geographic atrophy	Presence of geographic atrophy anywhere in the macular area that is not contiguous with the main lesion	• At baseline • Prompt at each clinical visit to check for change	Clinical data
	Subretinal fibrosis	Presence of subretinal fibrosis anywhere in the macular area	• At baseline • Prompt at each clinical visit to check for change	Clinical data
	Pigment epithelial detachment	Presence and type of pigment epithelial detachment anywhere in the macular area	• At baseline • Prompt at each clinical visit to check for change	Clinical data
Associated clinical history	Previous macular degeneration treatment	Previous macular degeneration treatment in affected eye (multiple options possible)	• At baseline	Clinical or administrative data
	Ocular comorbidities	Ocular comorbidities including retinal vascular disease, other macular pathology, glaucoma or optic neuropathy, amblyopia, or medial opacity in affected eye (multiple options possible)	• At baseline • Prompt annually to check for change	Clinical or administrative data
Additional interventions to be recorded	• Cataract surgery • YAG laser capsulotomy • Retinal laser (ie, for macular edema or diabetic retinopathy) • Vitrectomy • Corneal surgery (ie, graft, pterygium)		• Prompt at each clinical visit to check if any other interventions have occurred	Clinical or administrative data

has validated translations from the original English version into a number of languages including Chinese,[29] German,[30] Melanesian,[31] Hindi, and Telugu.[32] We recognize that widespread adoption will also require additional translations to ensure comparability across populations. The developers of the IVI are willing to provide advice on the process of translation and validation if needed.

Disutility of Care Outcomes. The burden of care to patients was deemed to be important to measure, as the frequent and regular treatments could have a negative impact on the lives of patients and of those who care for them, in terms of time and money as well as emotionally. It is also possible that the number of treatments received may not be the same as the number that would be recommended if the logistics of clinic capacity and frequent patient visits were not an issue or if there were no financial constraints. We therefore recommend that each individual treatment received for macular degeneration should be recorded at each clinical visit.

Complications related to treatment received for macular degeneration, although occurring infrequently, are of significant concern to patients. The most serious complication of intravitreal injections is endophthalmitis. We therefore recommend capturing the incidence of severe intraocular inflammation within 3 months of the intraocular treatment due to infectious or noninfectious causes. This outcome is applicable for all patients undergoing treatment for neovascular macular degeneration.

Other complications of treatment, such as retinal pigment epithelium tears and retinal detachment, were considered by the working group. However, they were rejected from this minimum outcome set on the basis that these were idiosyncratic complications, with potential variability in detection rates. Further information on the rationale of the working group decisions is included in Supplemental Table 1.

Disease Control Outcomes. Long-term control of disease was judged to be both significant to patients and often a reflection of the treatment provided. For all types of neovascular macular degeneration, we recommend reporting at each clinical visit the presence of intraretinal or subretinal fluid or hemorrhage that is attributable to activity of the neovascular lesion as determined by the treating ophthalmologist (based on clinical examination or imaging).

Clinical measures such as central retinal thickness or size of lesion were not included because these were not felt to be outcomes of treatment that were important to patients. A summary of all the outcomes that are recommended to be recorded is presented in Table 2.

• **CLINICAL CHARACTERISTICS AND INTERVENTIONS:** In order to make meaningful outcome comparisons between patients, it is important to measure certain baseline and follow-up characteristics to enable subsequent appropriate risk adjustment. The full list of potential clinical characteristics that were considered are provided in Supplemental Table 2 (Supplemental Material available at AJO.com), along with the final voting decisions of the working group on inclusion or exclusion of individual clinical characteristics. A number of clinical characteristics where there is good evidence of their impact on clinical outcomes following treatment for macular degeneration were discussed and finally selected.[33,34] These are summarized in Table 3, grouped under the categories of patient demographics, baseline functional status, clinical status, and associated clinical history. Systemic comorbidities such as cerebrovascular and cardiovascular disease were not included to minimize data collection burden. Similarly, the suggested reporting format for these characteristics and interventions has been simplified to facilitate data collection while still providing useful information.

We also recommend recording of interventions to the affected eye that are likely to have an impact on the outcomes that are recommended to be collected, in particular visual functioning and vision-related quality-of-life outcomes. These are also listed in Table 3.

• **DATA COLLECTION:** The timeline for the suggested recording of the different categories of outcomes and clinical characteristics is summarized in Figure 3. We also suggest prompts at each clinical visit to check if certain clinical characteristics have changed and if any other relevant interventions have occurred, as indicated in Table 3. We envisage that this would be performed by some form of electronic patient record and that the prompting, rather than have more mandatory fields, would reduce data collection burden.

A very important long-term goal of ICHOM is to produce data that can be easily compared across providers, centers, and countries. To achieve this, we recommend processes to reduce variability, including the use of similar data sources, recognizing that the specific details of data collection will differ by center. As outlined in Tables 2 and 3, the potential sources are administrative data, clinical data, and patient-reported sources. We recommend that the source of data, as well as the response rate (if patient reported), be tracked for every measure. In the face of regulatory, privacy, and information technology challenges, we advocate that centers without a national registry track these data individually with the anticipation that future efforts led by ICHOM will facilitate standardized comparisons among centers.

DISCUSSION

THE ICHOM MACULAR DEGENERATION WORKING GROUP set out to develop a *minimum* set of standardized

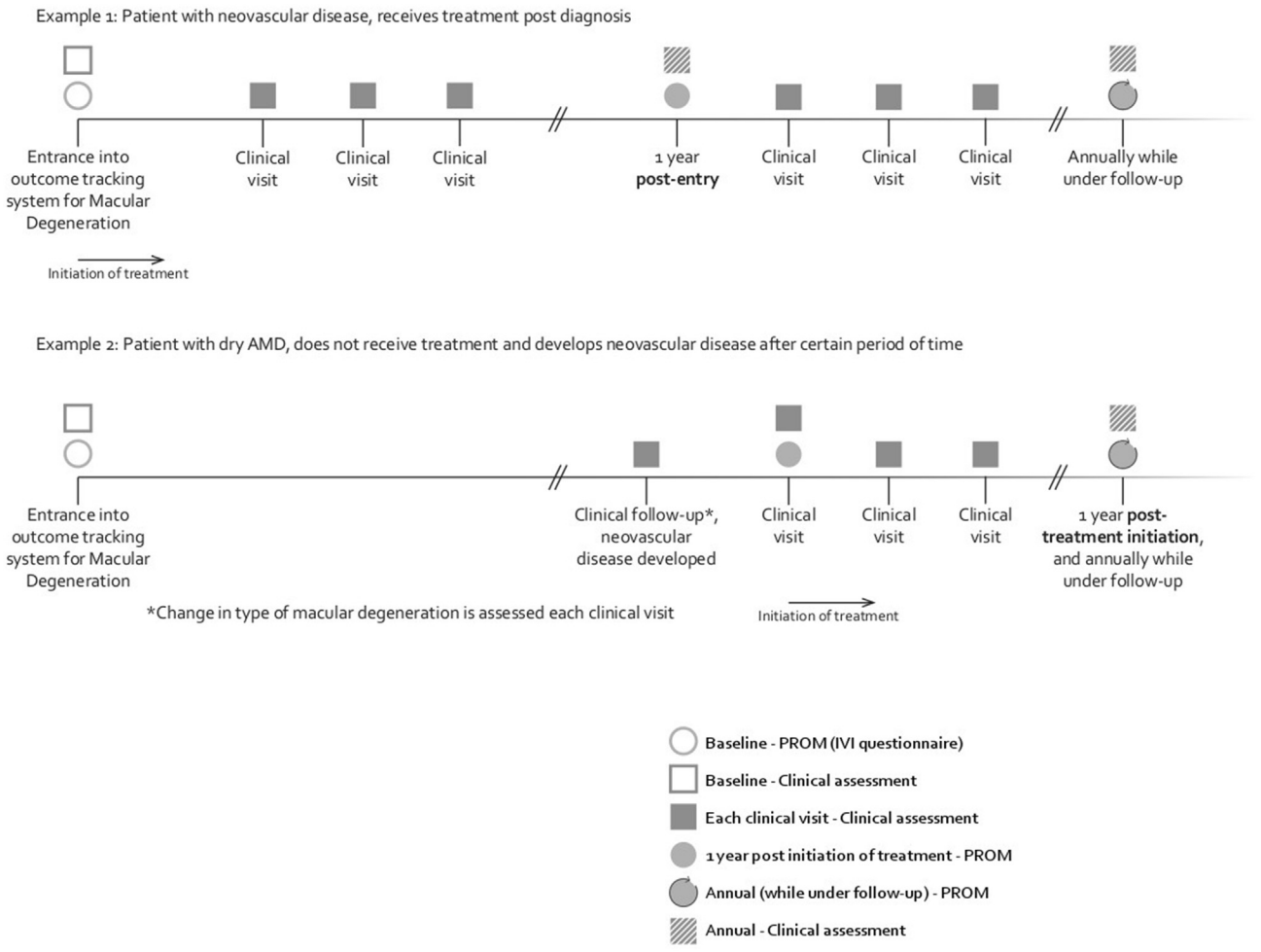

FIGURE 3. Time points for the suggested recording of outcomes and baseline characteristics in the International Consortium for Health Outcomes Measurement macular degeneration Standard Set.

patient-centered outcome measures for macular degeneration that are practical for all health care providers to record *in routine clinical practice*. Additional clinical characteristics are also suggested to ensure that it will be possible to perform case-mix risk-adjusted analysis. Although there are a number of existing projects where data on macular degeneration outcomes are routinely collected, our research showed that there are significant differences both in the outcomes that are collected and in the way they are measured. Defining a standardized set of minimum outcomes will therefore enable meaningful results for health care professionals to objectively assess their performance and drive improvements in their clinical practice. It will also allow for insurers or commissioners to understand the quality and value of care that is being funded, and—importantly—for patients to make well-informed decisions about their treatment.

According to the disease model suggested by Porter,[35] visual function in real-life situations, the burden and complications of receiving treatment, and long-term disease control and quality of life are likely to be of most significance to patients. On the other hand, change in central macular thickness or the size of the lesion have less direct impact on patients, yet are often reported and discussed. The macular degeneration outcome set that we developed includes a PROM. Although PROMs are used much less often than measurements of visual acuity, implementing regular PROM measurement as part of routine clinical ophthalmology practices is possible.[36]

Of course, measures included in the Standard Set reflect a balance between meaningful and pragmatic data collection that can be incorporated into existing patient pathways internationally, and in a variety of clinical contexts. The resultant minimum core dataset is therefore inevitably a compromise between intricate details that may be useful for comparison and the practicalities and burden of data collection. Interested care providers should therefore add additional outcomes to meet their specific requirements.

Implementation of the ICHOM macular degeneration Standard Set to collect data from routine clinical care

remains a key challenge for the future. The working group has a wealth of experience in outcomes data measurement and has designed this minimum dataset to be realistic to capture in most settings. The majority of the data is already measured in routine clinical practice, although not currently in a standardized manner. Furthermore, there are already existing projects that have produced numerous publications, including those by Gillies and associates[24] and Johnston and associates,[37] that use outcome data captured during routine clinical practice. The one exception to this is the PROM. However, the working group, particularly with the contribution of its patient representatives, believes that these patient-reported outcomes are as vital as any of the clinical outcomes. This is because only the patient-reported outcomes can truly reflect how a treatment actually affects patients. Furthermore, PROMs have demonstrated clinically relevant improvements in patient-reported visual functioning in randomized clinical trials of intravitreal therapy for neovascular macular degeneration regardless of whether the treated eye is the better- or worse-seeing eye at onset of treatment.[38]

We do also recognize that in many countries, significant financial and logistical challenges to collecting outcomes remain in terms of both clinical and patient-reported data. However, with future development in health information technology it is likely that these barriers will become less significant over time. ICHOM will continue to work with organizations around the world to encourage adoption of the macular degeneration Standard Set and is planning to facilitate global comparisons between provider organizations as adoption takes place.

We acknowledge that this work does have limitations. The proposed minimum dataset has not been widely consulted on and has been derived from consensus opinions. However, the working group has made these recommendations based on the best currently available relevant evidence and an enormous collective wealth of experience. Secondly, the dataset has not been piloted, and although we do not envisage problems with the feasibility of the clinical data collection (as the vast majority of the outcomes and baseline characteristics that are recommended have individually been used successfully in other registries and clinical trials), measurement of the PROM data may be a barrier to implementation, as this is not currently measured routinely. We anticipate that participation in data collection and feedback will inform future iterations of the Standard Set, and a steering committee will also be formed from the existing working group to review and, if necessary, update the recommendations on an annual basis, to ensure that it remains current and relevant for both patients and physicians with the developments in the evidence base and macular degeneration treatment.

We acknowledge that randomized studies remain the gold standard for outcome comparisons between treatments, but we believe that registries serve as essential companion efforts to assess the effectiveness of treatments in real-world settings. Nevertheless, the current work is also limited by the fact that the utility of this proposed minimum dataset is currently undetermined. It is likely, however, that collecting and reporting these data will lead to better understanding of the health benefits and prognosis for real-life clinical scenarios, as has been demonstrated in other areas of medicine and subsequent improvements in outcomes. This will be especially relevant for patients with multiple comorbidities who would usually be excluded from randomized clinical trials. The usefulness of the data will, however, be limited by the honesty in which it is collected the accuracy with which it is recorded and the transparency in which it is shared. It is important that this is emphasized, especially when outcomes are increasingly being linked to reimbursement.

High volumes of good-quality data from routine clinical practice can rapidly be achieved through systematic data collection of standardized outcomes. This provides new information that can enable better decision making about how, when, and where to treat patients with different clinical characteristics. It may shed light on the as-yet-unanswered and ever-growing questions in macular degeneration, including the optimal criteria for treatment, which treatment or combination of treatments, or which follow-up and retreatment regimen leads to the best outcomes that matter most to patients.

The United Kingdom Cataract National Dataset is an example in ophthalmology of how pooled data, collected as part of routine clinical care via electronic patient record systems, has provided valuable benchmarking standards that have later led to improvements in risk profiling of patients.[39] To help similar projects globally collect data in a standardized and therefore comparable manner, ICHOM has recently published a proposed minimum set of outcome measures for cataract surgery.[40]

We hope our recommendations will facilitate and accelerate global improvements in the outcomes of treatment of macular degeneration, by encouraging wider collaborative and consistent measurement of meaningful outcomes by care providers and enabling comparison between different care providers and different countries.

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