Effectiveness Guidance Document (EGD) Process

Updated June 2014
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EFFECTIVENESS GUIDANCE DOCUMENTS (EGDS)
EGDs provide clear, specific, and actionable recommendations on how to design and conduct research to generate the evidence needed by post-regulatory decision-makers, including patients/consumers, clinicians, and payers.

Recommendations provide guidance on specific design aspects (e.g., type of patient, outcome measures, follow-up intervals) that should be considered in order to ensure that future studies provide the most informative evidence possible for post-regulatory decision makers.

Unique to EGDS is GPC’s multi-stakeholder process which includes clinical researchers, product developers, patients/consumers, clinicians, and payers.

ROLES
The Consortium steers topic selection and recommendation development within a specific disease area. Consortium members provide input, specific stakeholder perspectives, and feedback throughout the entire EGD process, from topic selection to dissemination of the recommendations.

The Consortium Lead provides leadership to the Consortium and works closely with the TWG Chair to guide and steer the EGD process, to ensure that TWG members are and stay engaged throughout the process, that the perspectives of all stakeholder groups are solicited and considered, and that the Recommendations are specific, feasible, and likely to have a substantial impact on research.

The TWG Chair serves as the content expert for the EGD, provides suggestions for TWG membership, and is responsible for writing the Recommendations and EGD, with the TWG members.

The TWG members provide expertise and specific stakeholder perspective to draft the recommendations. TWG members work on individual recommendations depending on their expertise, and provide feedback on recommendations drafted by other TWG members.

GPC staff perform background research and provide synthesized results of this research for topic selection and for specific issues throughout the EGD process; participate in and summarize TWG discussions; and write sections of the EGD. GPC staff ensure the final Recommendations and EGD are evidence-based.

GPC is responsible for the content of the final Recommendations and EGD.

PROCESS
The general process for developing EGDS, and the resulting products from each step, is summarized in a flow chart in Appendix A and the specific steps described below. Although these are presented as linear steps, the tasks may be iterative and there is flexibility in how the steps are completed.

Step 1. Topic Identification and Selection
Product: Topic

In short, topics should address an evidence gap, be timely, feasible to address through methodology, and potentially impactful for decision-makers if solutions for evidence generation are successfully identified.
The following factors should be considered for topic selection.

- Significant public health impact, unmet need, and/or cost.
- Significant variability in evidence expectations across payers.
- Conflict between regulatory requirements and evidence needs of other decision makers (e.g. accelerated approval, breakthrough classification).
- Major tensions between ideal methods and feasibility. For example, randomization or blinding are not feasible, or intermediate outcomes are used because long-term outcomes are not feasible.
- Developing recommendations should not duplicate ongoing public/private sector work but rather complement it.
- Specific opportunities to improve research are identifiable, that is, recommendations can be developed that are feasible, and that, if followed, will have a substantial impact on the quality and relevance of research in the topic area.

In order to identify topics that are most relevant to stakeholders, GPC may conduct key informant interviews with appropriate experts. Input from the Consortium may be obtained in a number of ways, including interaction through the SharePoint space, online questionnaires, discussion at in-person Consortium meetings, personal interviews, and/or small group (Consortium sub-group) discussions by telephone.

As part of the topic generation process, GPC staff complete a brief review of relevant evidence-based guidelines, systematic reviews, and health technology assessments (see Appendix B). This step is essential to ensure that topics discussed by the Consortium have emerged from an evidence-based process. The purpose of this review is to identify or verify gaps in knowledge resulting from recurring methodologic deficiencies in the body of evidence—particularly the gaps that hinder decision-making by clinicians, patients, and payers.

The final topic selection is made by GPC.

**Step 2. Identification and Engagement of Technical Working Group (TWG)**

**Product: TWG**

The Technical Working Group (TWG) is formed to develop the topic and create recommendations to address the identified evidence gap.

First (or early in the TWG formation process), a **TWG chair** is selected on the basis of the following criteria:

- Recognized expertise relevant to the selected topic
- Clear interest in the topic and conviction that it is important to address
- Demonstrated leadership ability; preferably also a demonstrated ability to work in multidisciplinary teams
- Willingness to respect and take seriously various stakeholder perspectives, including those of patients and consumers
- Excellent writer
- Willing to commit to leading EGD and manuscript writing (with the support of the consortium team) (1 year, 5-10% of time; individuals who are in their early to mid-career may be most likely to be willing and able to commit to the responsibilities), but it is also important for the Chair to have a substantial amount of experience.

Next, the consortium Lead and Chair identify the **stakeholder experts** needed to populate the TWG to address the identified evidence gap. Nominations for TWG members are sought from the Consortium, and may also be made by the TWG Chair, GPC staff, gleaned from “snowballing” methods (i.e., referrals from prospective TWG members who elect not to participate) and solicited from the GPC Advisory Committee. An open call for nominations is made to one or more professional organizations to ensure a wide search and to prevent the limitations of an ‘old boy’ network. Nominations are reviewed by the consortium Lead and the TWG chair prior to invitation.

Experts are chosen based on demonstrated expertise in the field of interest, as exhibited by a record of relevant accomplishment. Balance of representation across stakeholder groups should be sought. Ideally, each TWG includes members of each of the following stakeholder groups: patients/consumers, payers, clinicians, payers, methodologists, industry-based researchers, academic researchers, and product developers.

Approximately 8 to 12 individuals compose the TWG. The number is determined largely by the types of expertise required for the topic. The group is kept relatively small, however, to promote cohesion and focus. While it is desirable to have a core group of experts established when initial meetings begin, TWG members may be added at any time as new forms of needed expertise are identified.

The Consortium lead works closely with the TWG Chair to guide the process, to ensure that TWG members are and stay engaged throughout the process, and that the perspectives of all stakeholder groups are solicited. Although much of the work may be conducted via email, teleconference, and webinar, an in-person meeting is typically scheduled early in the process to help create rapport among the group.

While all TWG members require preparation, special effort should be made to prepare patients for the topic to be discussed and the role they are expected to play. For highly technical subject matter where it would be unreasonable to expect advocates to “get up to speed” and technically skilled advocates are not available, patient representatives should be consulted on the best way to engage them for the purposes of that topic. In all cases, advocates should be clear on the nature of the problem to be addressed and the role they are expected to play on the TWG.

**Step 3. Topic Refinement**

*Products: Clinical Framework; State of evidence*

TWG members are oriented to the methods and purpose of EGDs. They participate in refining the topic by defining the scope of the problem statement, including the targeted clinical setting(s), identifying key issues and challenges associated with studying the problem and sorting technical from non-technical barriers to implementation. Decisions made on scope and setting are shared with the Consortium through the SharePoint space for comment and discussion, with the goal of arriving at a shared understanding of the final EGD scope.
While the state of evidence will already have been investigated to some degree in the topic selection process, more detailed elaboration of the evidence gaps associated with the topic may be required for focused recommendation development. The TWG may at any point during the process request additional background research. GPC staff will provide background research using any of the resources listed in Appendix A, results of focused literature searches, and review of specific research papers, as well as additional sources of information specified by TWG members.

We use the products from this step to form part of the draft version of the EGD, specifically for the Introduction sections “Clinical Framework” and “State of the Evidence” (see Appendix C).

Steps 4 and 4a occur in parallel.

**Step 4. Recommendation Development**

*Product: Recommendations*

Draft recommendations are developed by the TWG with guidance from the Chair and Lead, based on discussions that take place in TWG meetings, individual research and expertise provided by individual TWG members, input from Consortium members, and background research provided by GPC staff.

Recommendations, typically 10 to 13, are clear, specific, and actionable statements providing guidance on specific study design aspects (e.g., type of patient, outcome measures, follow-up intervals) that will ensure that future studies provide the most informative evidence possible for patients/consumers, clinicians, and payers (see Appendix B for further details).

If significant non-technical barriers exist to realistic implementation of the recommendations, the group may opt to include a number of non-methodologic “position statements” on policy changes or other actions needed to allow unimpeded evidence generation to take place.

Individual TWG members draft recommendations specific to their personal expertise and review and discuss each other’s work. The Lead focuses on the multi stakeholder process and the goal of the recommendations, while the Chair focuses on the content of the discussions.

Input from Consortium members is sought to ensure a thorough multi-stakeholder process. TWG discussion notes, drafts of recommendations for review, and other materials may be shared on the Consortium SharePoint space to allow Consortium members opportunities to review and comment on the developing recommendations. GPC staff members check the SharePoint discussion board regularly to see if Consortium discussion threads have developed relevant to the work of the TWG, and relevant comments and discussions are shared with the TWG. Periodic email reminders go to Consortium members to alert them that materials are available for review.

When the recommendations have been developed, Consortium members are asked to provide specific feedback using a questionnaire that focuses on the relevance, feasibility, and potential for impact of each recommendation.

Throughout the process, GPC staff should be prepared to conduct additional research or seek additional expertise for the group, as appropriate.
Step 4a. Dissemination Planning

When some clarity has been achieved on the problem statement and recommendations, the TWG should discuss potential opportunities for presentation, and possible interest of individual TWG members in submitting abstracts or panel proposals. TWG members may also be interested in creating manuscripts in addition to the one the Chair will author. TWG members should be encouraged to promote the project and their work on it, if interested in doing so. See also: Authorship under Step 10.

Step 5. EGD Development

Product: Draft EGD

In collaboration with the TWG chair, GPC staff drafts the EGD using the products of Step 3 and 4. For each recommendation an evidence-based rationale is provided, with suggestions, if needed, for how the recommendation may be implemented. See Appendix B, Outline for Effectiveness Guidance Documents, for details on the sections and information included in each section. In collaboration with the Chair, GPC staff draft the introductory material, assemble the drafted recommendations, and provide the full document to the TWG for final review before delivery to the consortium. At this stage, the GPC Program Director reviews the document. Other CMTP staff not involved in the writing may elect to review the document.

Step 6. Review of EGD

Product: Revised EGD

The draft EGD plus a survey with specific questions are distributed to the Consortium via the SharePoint working group space or via email. Consortium members are also encouraged to post comments at the SharePoint discussion board or to provide feedback privately by email to the Consortium team. GPC staff gather and organize all comments for TWG review and discussion. Individual TWG members, the TWG chair, and GPC staff make revisions, as appropriate, to address the concerns of the consortium.

Step 7. Consortium Meeting and TWG Meeting

Product: Revised EGD

GPC arranges a meeting or teleconference to review the revised recommendations with the TWG and discuss comments received. The TWG then reconvenes to agree on needed revisions. The TWG revises the recommendations based on the feedback.

Step 8: Posting

CMTP’s marketing team posts the EGD on CMTP’s website, announces its release in CMTP’s newsletter, and issues a press release.

Step 9. Non-consortium Peer Review

Product: Revised EGD

The EGD is sent to external peer reviewers selected from different stakeholder groups. GPC staff revise the EGD and produce the final EGD based on the feedback.
Step 10. Dissemination, Publication, and Updates
CMTP associates are alerted to the posting of the EGD by email, press release, and other communication channels.

A manuscript based on the EGD is typically submitted for publication in a peer-reviewed journal, and the results of the EGD process are presented at relevant scientific meetings. The Recommendations may be presented in a GPC webinar.

Authorship
The TWG Chair is usually responsible for preparing the manuscript and either the Chair or GPC staff take care of its submission. All members of the TWG and any additional individuals who have contributed to the development of the recommendations (as determined by the Lead) are mentioned in the manuscript. In addition, TWG members are offered an opportunity to be involved in the preparation of the manuscript and to be included as an author. GPC staff who have contributed to the content of the recommendations may be included as authors.

TWG members may develop manuscripts in addition to the one the TWG Chair authors. If the main focus of such manuscripts is a description of the EGD process or the resulting Recommendations, authorship should include appropriate GPC staff. However, if the material has been developed significantly beyond the original (e.g., report of a pilot study that uses the recommendations) a citation of the original publication, and reference to GPC in the body of the article, if appropriate, is sufficient.

A slide set describing the EGD is developed for TWG and consortium members to adapt and use in presentations. The slide set will also be posted on the website.

Updates
The public may provide comments on current EGDs through the CMTP website. GPC staff review comments, as well as new developments, to determine if an EGD should be updated or archived. All EGDs are reviewed for currency every 2 years. Outcomes of this review include decisions to update, to leave as is or to archive EGDs.
APPENDIX A: BACKGROUND RESEARCH

We consider the following sources for background research, as appropriate:

GENERAL RESEARCH GUIDANCE
- FDA guidance documents
- Methods guides for the Agency for Health Care Research and Quality (AHRQ’s) Evidence-based Practice Centers and DeCIDE centers
- Reporting standards, such as CONSORT and REMARK (equator network)

TOPIC SPECIFIC GUIDANCE
- Systematic reviews: The Cochrane Library, Evidence-based Practice Centers
- Evidence-based guidelines: National Guidelines Clearinghouse, Guidelines International Network
- National Institutes of Health consensus conferences and workshops
- Health technology assessments: National Institute for Health and Clinical Evidence, Canadian Agency for Drugs and Technology in Health, International Network for Health Technology Assessment, BlueCross Blue Shield Association Technology Evaluation Center
- Individual peer-reviewed research papers

The background research ensures the Recommendations and EGD are evidence-based.
APPENDIX B: EFFECTIVENESS GUIDANCE DOCUMENT FLOWCHART

Group

- GPC, with Consortium Input
- TWG, with Consortium Input, and GPC Staff
- TWG Chair, GPC Staff
- Consortium
- TWG
- Non-consortium Peer Reviewers
- GPC

Process

- Topic Selection
  - Priority Setting
  - Nomination
  - Background Research
  - Key Informant Interviews
- Topic Refinement
- Recommendation Development
- EGD Draft
- EGD Review
- Revisions
- EGD Review
- Revisions

Product

- Topic
- EGD Sections: Clinical Framework State of Evidence
- Recommendations
- Draft EGD
- Consortium Feedback
- Revised EGD
- Peer Review Feedback
- Final EGD

POSTING OF EGD