IQWiG Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen

Involvement of people affected in the dossier assessment[1]

[1] Translation of the document *Beteiligung von Betroffenen bei der Dossierbewertung* (Version 1.1; Status: 28 July 2017). Please note: This translation is provided as a service by IQWiG to English-language readers. However, solely the German original text is absolutely authoritative and legally binding.

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List of abbreviations

Abbreviation	Meaning
AMNOG	Gesetz zur Neuordnung des Arzneimittelmarktes (Act on the Reform of the Market for Medicinal Products)
EMA	European Medicines Agency
G-BA	Gemeinsamer Bundesausschuss (Federal Joint Committee)
IQWiG	Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen (Institute for Quality and Efficiency in Health Care)

1 Background

The early benefit assessment according to the Act on the Reform of the Market for Medicinal Products (AMNOG) became effective on 1 January 2011. This law requires pharmaceutical companies to submit a dossier for drugs containing new active ingredients to the Federal Joint Committee (G-BA) when these drugs enter the market. On the basis of studies, the dossier must show the added benefit of the new drug in comparison with the standard treatment ("appropriate comparator therapy"), specified beforehand by the G-BA. The dossier forms the foundation for the G-BA's early benefit assessment. This assessment must be completed within 3 months. In general, the G-BA commissions the Institute for Quality and Efficiency in Health Care (IQWiG) with the benefit assessment. After this assessment, a commenting procedure is conducted at the G-BA. The written commenting procedure, the oral hearing and the G-BA's resolution on the added benefit must be completed within another 3 months. If a drug has been proven to have an added benefit, price negotiations take place between the National Association of Statutory Health Insurance Funds and the pharmaceutical company. If the parties cannot agree on a discount on the price originally set by the company, an arbitration board determines the reimbursement price (Figure 1).

Due to legal regulations, patient organizations are involved in the entire AMNOG procedure. At the G-BA, this applies to the collaboration in different committees, the commenting procedure and the possible arbitration board.

Although there is no corresponding legal obligation for IQWiG with regard to dossier assessments, as in its other commissions, it is particularly important for IQWiG to ensure the involvement of affected people[2] so that the patient perspective can be considered in the assessment.

The following text only refers to their involvement in IQWiG's dossier assessments.

[2] "People affected" can in particular be patients (if necessary, represented by their parents or other relatives) as well as potential participants in preventive measures.

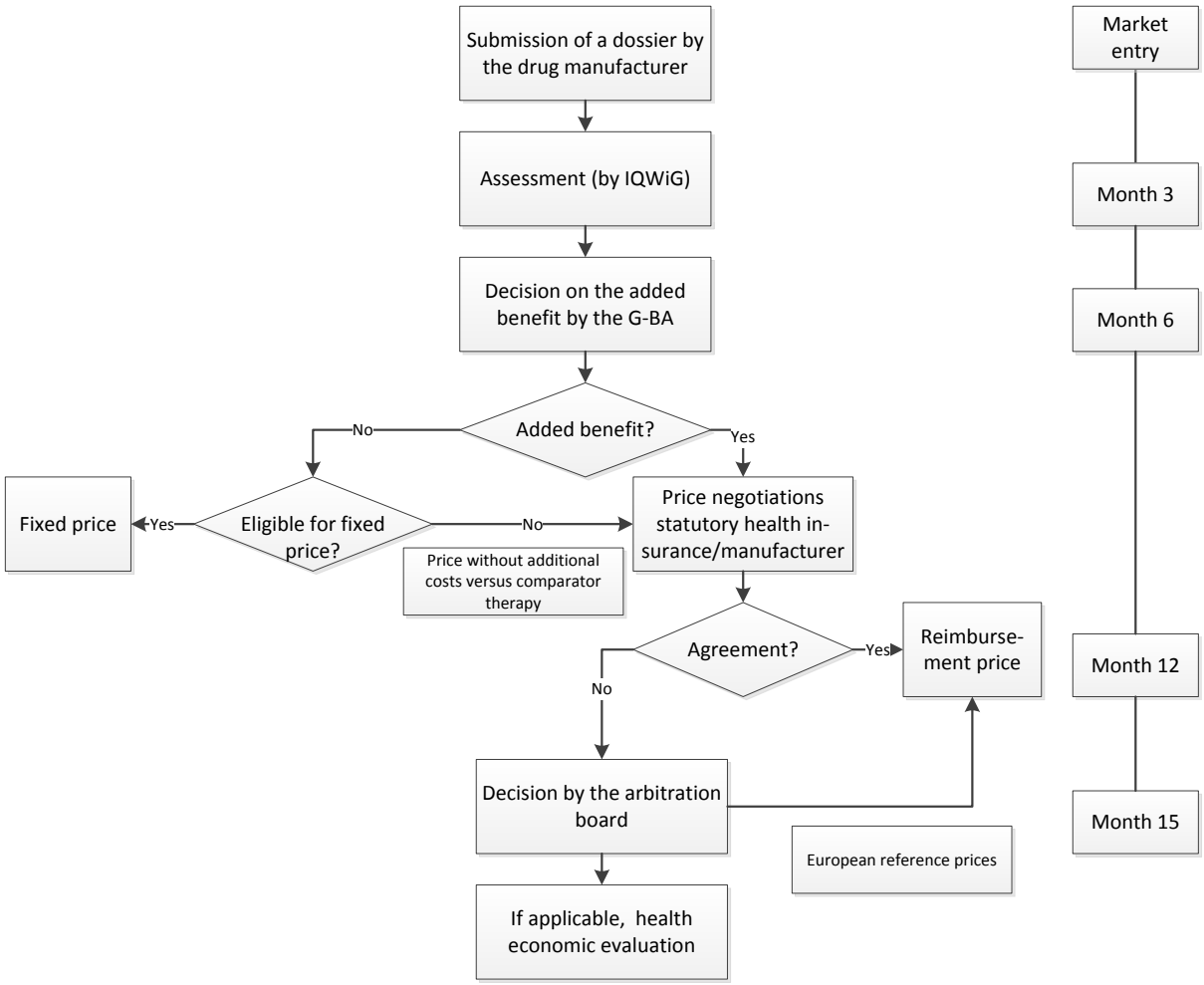

Figure 1: Procedure of the early benefit assessment of drugs containing new active ingredients

2 Procedure of patient involvement at IQWiG

2.1 Type of involvement of people affected

The procedure of the involvement of people affected is characterized by the narrow time frames for a benefit assessment. For this reason, no personal interviews can be conducted with people affected, but specific questionnaires are used. In the development of the questionnaire, patient representatives were consulted within the framework of two events.

2.1.1 Questionnaire

The questionnaire developed specifically for the dossier assessment (Appendix A) contains the following sections:

- Administrative part
  - information on the drug
  - contact details of the person completing the questionnaire
  - declaration of consent for the publication of information
  - declaration of potential conflicts of interest
- Questions
  - information on the disease
    - experiences with the disease
    - necessity to consider specific patient groups
  - information on the treatment of the disease
    - experiences with the treatments currently available for the therapeutic indication
    - expectations for a new therapy
  - additional information
- Questions and answers for the consideration of the patient perspective

The information on the drug (name of the active ingredient, trade name if known, therapeutic indication and link to documents of the European regulatory authority, the European Medicines Agency [EMA]), aims to provide the person completing the questionnaire with a better overview of the drug under assessment. The contact details have been included for queries regarding the information provided in the questionnaire.

The key part of the questionnaire and the actual participation of people affected are the questions on the disease and its treatment. The main question is supported with further detailed questions, which do not have to be answered individually. They are only included to make the respective question more specific.

Answering the questions can provide information on the appropriate comparator therapy, patient-relevant outcomes, side effects of particular interest, patient subgroups and other aspects from the point of view of people affected.

The questionnaire also contains questions and answers to clarify the framework for the consideration of the patient perspective and provide further information.

2.1.2 Conflicts of interest

People who complete the questionnaire are also required to disclose information on potential conflicts of interest. For this purpose, they complete the general form for conflicts of interest, which is also used for other IQWiG products.

The declared conflicts of interest are published together with the dossier assessment. However, it is only stated whether or not there are conflicts of interest in the area covered by the question, i.e. only the answer "yes" or "no" is provided. No specific details on relationships or the amount of any remuneration received are published.

2.1.3 Declaration of consent for the publication of information

The declaration of consent to publish information provided in the questionnaire also forms part of the questionnaire.

2.2 Selection of people affected

People affected are contacted via the spokesperson of the Coordination Committee of the relevant patient organizations, who is also responsible for the coordination of the G-BA patient representatives[3].

2.3 Time flow

The questionnaire with information on the drug and the form of conflicts of interest are sent to the spokesperson of the Coordination Committee of the relevant patient organizations. The spokesperson passes on the documents to the corresponding patient organizations. Starting from transmission of the documents, they have 15 working days to complete the questionnaire and the form of conflicts of interest and submit them to IQWiG electronically or by post. For legal reasons it is necessary to submit the signed original documents. These documents must be submitted within 10 further working days (Figure 2).

[3] At the G BA, only certain organizations are entitled to take part in discussions and submit petitions. The criteria for approval are stipulated in the Patient Involvement Regulation. Besides further criteria, in particular the disclosure of the funding of the organizations and hence proof that the organizations work in a neutral and independent way are required for their recognition.

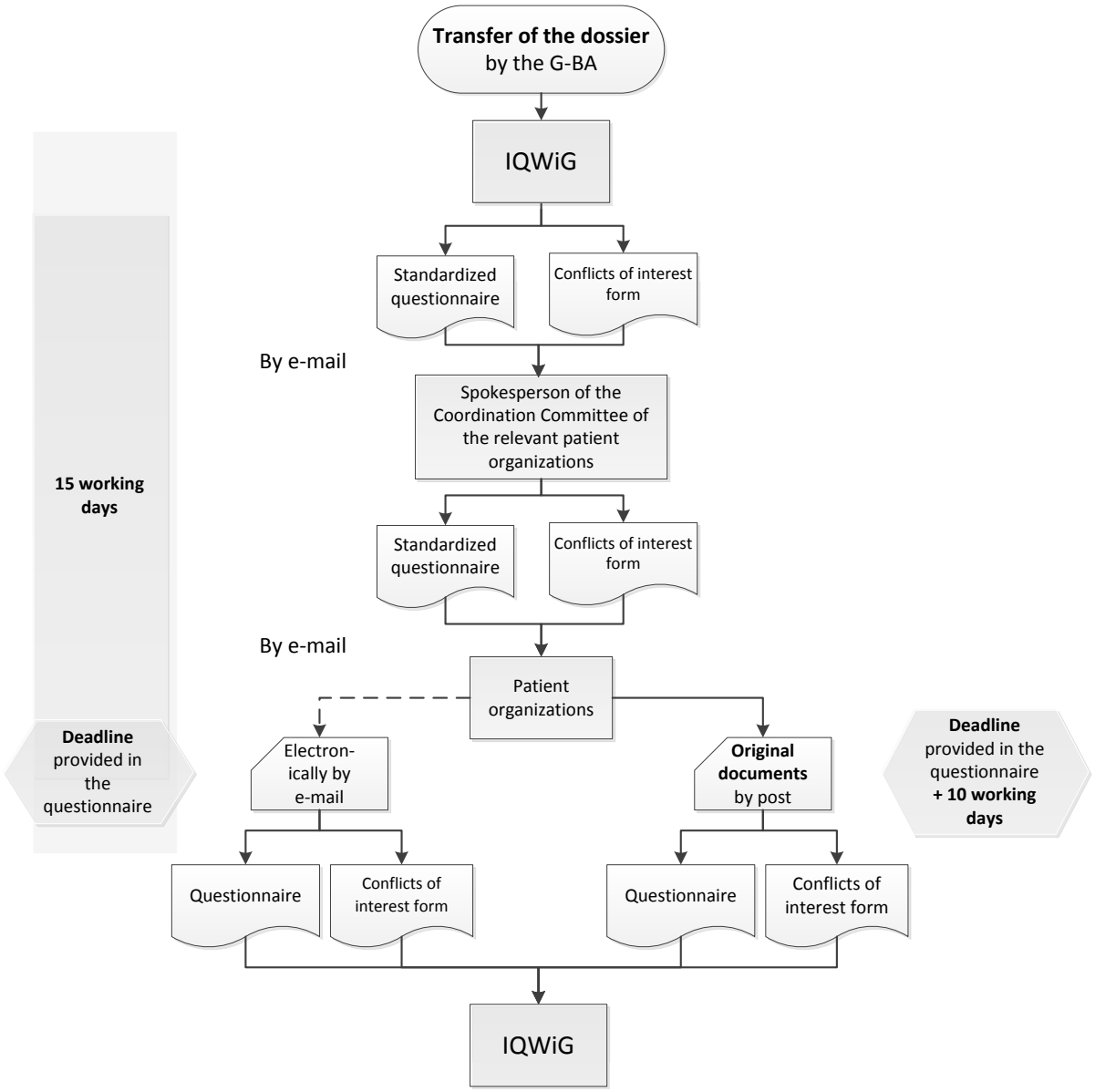

Figure 2: Process of involvement of people affected in the dossier assessment

3 Further aspects

On request, people affected receive feedback on their completed questionnaire. To receive feedback, they can contact IQWiG after publication of the dossier assessment. Questions on the questionnaire or the procedure are also answered.

There is no specific training on how to complete the questionnaire, but workshops on patient involvement are conducted at IQWiG at irregular intervals.

No remuneration or financial expense allowance is paid for participation.

Appendix A – Questionnaire for involvement of people affected in the early benefit assessment

IQWiG Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen

Institute for Quality and Efficiency in Health Care

Involvement in IQWiG's early benefit assessment

Questionnaire for the description of a disease and its treatment for people affected and patient organizations

(Status: January 2017)

1 General information

Within the framework of the Act on the Reform of the Market for Medicinal Products (AMNOG), drugs containing new active ingredients undergo an early benefit assessment on the basis of dossiers. The respective dossier is compiled by the relevant pharmaceutical company. The aim of this procedure is to determine within 6 months which added benefit of new drugs is proven in comparison with an appropriate comparator therapy. The results of this benefit assessment are the basis for drug pricing. In addition, they also provide important information for people affected and physicians.

The production time of IQWiG's early benefit assessment (dossier assessment) is legally limited to 3 months. To consider the perspective of people affected in the dossier assessment, IQWiG developed this questionnaire to pass on information on the disease and its treatment. The answers provided by people affected and patient organizations are considered in the dossier assessment. It is necessary to receive feedback shortly after the start of the process. The perspective of people affected might also be provided by relatives (e. g. parents) or patient representatives.

Please support us by providing your knowledge on the disease and its treatment, complete the questionnaire for project:

Project number: <completed by IQWiG>

from the point of view of people affected, and return the completed questionnaire to IQWiG by

Deadline for submission: <completed by IQWiG >

either by e-mail to arzneimittel@iqwig.de or, as the original document, to the following address:

IQWiG
Ressort Arzneimittelbewertung
Stichwort „Dossierbewertung"
Im Mediapark 8
50670 Köln

You are also required to send the completed Form for disclosure of potential conflicts of interest. You have been sent this form together with this questionnaire.

If you submit your documents by e-mail first, please send the original documents (with your signature) to the above address within 10 working days after the deadline mentioned above, i.e. by **<completed by IQWiG>** at the latest.

Please note that only information submitted within the deadline can be considered in the dossier assessment. You can find more details on this and a list of frequently asked questions at the end of this questionnaire.

1.1 Information on the drug

Below you will find some information on the drug assessed by IQWiG.

Active ingredient:
<completed by IQWiG>

Trade name (if already known):
<completed by IQWiG, if not yet known entry: “Not yet known”>

Therapeutic indication:
<easily understandable text from the EPAR version for the public or from the Summary of Product Characteristics; completed by IQWiG>

Link to documents of the European Medicines Agency (if applicable):
<completed by IQWiG, either corresponding link to EPAR + EPAR version for the public or entry “EMA has not yet published any documents”>

EPAR - assessment report of the European regulatory authority:
<completed by IQWiG>

EPAR version for the public:
<completed by IQWiG>

1.2 Contact details

Please fill in your contact details and, if applicable, cite the patient organization on behalf of which you are completing this questionnaire.

Name (for queries):

E-Mail address:

Phone number:

Name of patient organization:

Position in this patient organization:

Website:

1.3 Declaration of agreement

The following declaration of agreement is required to be able to publish your information within the framework of the dossier assessment. Please enable us to use this information by providing the necessary signature.

Declaration of agreement: I/we know that the name of the patient organization (if applicable) and all information provided in Section 2 to 4 of this questionnaire may be published on the Internet within the framework of the IQWiG report on the dossier assessment.

Place / Date:

Signature:

1.4 Declaration of potential conflicts of interest

Please complete the *Form for disclosure of potential conflicts of interest* and submit it together with the completed questionnaire.

You can find explanations on this form under https://www.iqwig.de/en/participation/conflicts-of-interest/frequently-asked-questions-about-the-form-for-disclosure-of-potential-conflicts-of-interest.3307.html.

2 Information on the disease

2.1 Disease-related experiences of patients and people affected

What impairments and other aspects occur in daily life in relation to the disease for this therapeutic indication, affecting quality of life, among other things?

The following questions are meant to help you formulate your answer. It is not necessary to answer each individual question.

Possible aspects for answering the question may be:

(1) Which aspects and symptoms of the disease are more important to treat or to control than others?
(2) How does the disease affect your daily life (job, family, spare time) or that of people affected?
(3) How does the disease influence your job situation or that of the patients?
(4) Are there any activities you or people affected are not able to perform because of the disease?
(5) If the disease lasts for a longer period of time: Is there anything important to consider in the course of the disease?
(6) What are the challenges in the care of people affected with this disease?
(7) How does the treatment affect the daily routine of patient care?

Enter your answers to question 2.1 here

2.2 Necessity to consider specific patient groups

Is it important to consider specific patient groups?

The following questions are meant to help you formulate your answer. It is not necessary to answer each individual question.

Some examples of different patient groups:

(1) Are there important differences between men and women?
(2) Are there important differences regarding younger and older patients?
(3) Are there important differences regarding different phases of the disease?
(4) Are there differences regarding ethnic groups?

Enter your answers to question 2.2 here

3 Information on the treatment of the disease

3.1 Experiences of people affected with the treatments currently available for the therapeutic indication

How good can you or the people affected manage their condition with the treatments known to you? These may include both drugs approved in Germany and drugs not approved for the therapeutic indication of the new drug - so-called “off-label” use. Please provide your answers referring to the therapeutic indication mentioned in Section 1.1.

The following questions are meant to help you formulate your answer. It is not necessary to answer each individual question.

Some aspects that may be relevant:

(1) Which treatments do you or people affected currently use for treating the disease in the therapeutic indication?
(2) How effective is the current therapy in treating the disease?
(3) How does the treatment affect different aspects of life (job, family)?
(4) Are there any side effects that are more difficult or easier to tolerate than others?
(5) Are there any important issues in the course of the treatment of the disease?
(6) Based on the experience of some or several people affected, is there a need that is not met by current treatment? Which one? Does this affect a certain group of patients in particular (e.g. men/women)?

Enter your answers to question 3.1 here

3.2 Expectations for a new therapy

What do you or people affected expect from a new therapy? Besides drug treatments, you may also take into account non-drug therapies. Please provide your answers referring to the therapeutic indication mentioned in Section 1.1.

The following questions are meant to help you formulate your answer. It is not necessary to answer each individual question

Examples of possible aspects:

(1) Which problems (e.g. side effects) do you know that can occur under current therapies and that should be addressed with a new therapy?
(2) Is there a specific gap in current therapy that should be closed with the new therapy?
(3) Which side effects are acceptable, and which are not?
(4) What would you or people affected expect regarding the use of the new therapy?
(5) Are there any expectations regarding the form of administration of a new treatment?

Enter your answers to question 3.2 here

4 Additional information

Is there any other information that you would like to impart to IQWiG (optional)?

Enter your answers to question 4 here

5 Question and Answers

Below you will find questions and answers on the consideration of the patient perspective in IQWiG's dossier assessments.

5.1 How are people affected and patient organizations involved in IQWiG's early benefit assessments?

On receiving the commission from the Federal Joint Committee (G-BA), IQWiG sends the present questionnaire to the spokesperson of the Coordination Committee of the relevant patient organizations according to §140f SGB V. The spokesperson passes the questionnaire on to the patient organizations that represent the patients affected by the disease.

5.2 How much time do the people affected or patient organizations have to complete the questionnaire?

The patient organizations have 15 working days to complete the questionnaire, starting from the transmission to the spokesperson of the Coordination Committee of the relevant patient organizations according to §140f SGB V. The deadline for submission is noted in the questionnaire.

The date of receipt at the Institute is decisive for submission within the deadline. The timely receipt by e-mail is initially sufficient to meet the deadline. Then the original documents must be submitted within 10 working days of the deadline. Another option is to directly submit the original documents by the deadline; in this case no submission by e-mail is required. The person submitting the questionnaire is responsible for the timely receipt of the completed questionnaire by the deadline, and of the original documents within 10 working days after the deadline at the latest.

5.3 What other prerequisites must be met?

To consider your information you are additionally required to fully complete the Form for disclosure of potential conflicts of interest and submit it within the deadline.

You can find explanations on this form under
https://www.iqwig.de/en/participation/conflicts-of-interest/frequently-asked-questions-about-the-form-for-disclosure-of-potential-conflicts-of-interest.3307.html.

The connections you have disclosed in the form will be published within the framework of the dossier assessment. Only the types of connections that exist or do not exist are listed in a table. No specific details on business partners or the amount of any remuneration received are published.

5.4 Where do I send the completed documents?

Please use the following address for all documents sent by e-mail: arzneimittel@iqwig.de. Please send original documents to the following address: IQWiG, Ressort Arzneimittelbewertung, Stichwort „Dossierbewertung", Im Mediapark 8, 50670 Köln.

5.5 Should I also send the completed questionnaire to the BAG Selbsthilfe (Federal Self-Help Association)

It is very helpful for the G-BA patient representatives and for the BAG Selbsthilfe staff to receive the information provided in this questionnaire. If you wish to support their work, please send the completed questionnaire to the following e-mail addresses:

- patientenbeteiligung@g-ba.de
- geschaeftsfuehrer@bag-selbsthilfe.de

5.6 Should I enclose the literature cited?

If you cite literature, it would help us if you also provided this literature to us via e-mail. However, this is not mandatory.

5.7 How can I receive feedback on my completed questionnaire?

If you wish to receive feedback on your completed questionnaire, please send an e-mail to arzneimittel@iqwig.de. We will contact you after completion of the dossier assessment.

5.8 Do you have any further questions on the procedure or the content of the questionnaire?

Please contact arzneimittel@iqwig.de for any questions on the procedure or the content of the questionnaire.