IQWiG Reports – Commission No. H21-08

# Transcranial magnetic resonance-guided focused ultrasound (tcMRgFUS) for the treatment of essential tremor

## 2nd addendum to commission H20-05[1]

## Extract

[1] Translation of the executive summary of the addendum H21-08 *Transkranialer Magnetresonanz-gesteuerter fokussierter Ultraschall (TK-MRgFUS) zur Behandlung des essenziellen Tremors – 2. Addendum zum Auftrag H20-05* (Version 1.0; Status: 9 July 2021). 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.

# Publishing details

**Publisher**
Institute for Quality and Efficiency in Health Care

**Topic**
Transcranial magnetic resonance-guided focused ultrasound (tcMRgFUS) for the treatment of essential tremor –2nd addendum to commission H20-05

**Commissioning agency**
Federal Joint Committee

**Commission awarded on**
27 May 2021

**Internal Commission No.**
H21-08

**Address of publisher**
Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen
Im Mediapark 8
50670 Köln
Germany

Phone: +49 221 35685-0
Fax: +49 221 35685-1
E-mail: berichte@iqwig.de
Internet: www.iqwig.de

**IQWiG employees involved in the addendum**

- Andrea Steinzen
- Ralf Bender
- Elke Hausner
- Stefan Sauerland
- Sibylle Sturtz

**Keywords:** High-Intensity Focused Ultrasound Ablation, Magnetic Resonance Imaging, Essential Tremor, Device Approval, Risk Assessment, Benefit Assessment

## Executive summary

In a letter dated 27 May 2021, the Federal Joint Committee (G-BA) commissioned the Institute for Quality and Efficiency in Health Care (IQWiG), as an addendum to commission H20-05, to describe possible key points of a registry-based, non-randomized study to supplement the randomized testing study.

### *Research question*

With regard to tcMRgFUS for treating drug-refractory essential tremor in patients eligible for deep brain stimulation, the aim of the present investigation was to

- describe possible key points of a registry-based non-randomized study

to supplement the randomized testing study outlined in the §137h assessment H20-05.

### *Key points of a registry-based study*

An outline for a registry-based study to supplement a randomized testing study is developed against the background that the condition of interest is extremely rare. This leads to a pragmatic approach in some aspects of study design. The key points of the registry-based study include the recommendation to develop a study with a prospective design. The research question (population, intervention, comparator, outcomes) is the same as that of the randomized testing study outlined in H20-05. Essential components of the study design are a detailed study protocol, finalized before the start of data collection, which includes in particular the prespecification of possible confounders and details on the scope and duration of data collection.

No potentially suitable registry could be identified via a search for disease registries on essential tremor. Therefore, the establishment of a new registry is necessary for data collection. The time needed to set up such a new registry is estimated to be about 1 to 4 years, depending on various factors. Quality criteria for disease registries are listed, with the goal of achieving the highest possible outcome quality

### *Prospects of success*

A registry-based study that is suitable to support the results on potential effects observed in the randomized testing study is possible in principle. The implementation of such a registry-based study is resource-intensive, among other things because no suitable registry is known so far and the establishment of a new registry is therefore necessary. Due to the extreme rarity of the condition, the design of the study will have to follow a pragmatic approach in some aspects. Nevertheless, after a few years, the results could be used to support the results on potential effects observed in the randomized testing study. In the event that the randomized testing study recruits fewer patients than expected, and thus ultimately not enough, such a registry-based study could at least serve as rough orientation for a guidance decision at the end of the testing study. The informative value will depend primarily on how many patients are ultimately

included in the registry. Compared to the status quo, the randomized testing study and the outlined supplementary registry-based study on the currently extremely rare condition would create the best data basis possible under the given circumstances in Germany.

One possibility to include more patients in the registry-based study and thus to increase validity is the option to extend the study to an international study.

The full report (German version) is published under

*https://www.iqwig.de/en/projects/h21-08.html*