Appendix M: Call for evidence

A call for evidence was issued to identify unpublished data of relevance to review questions on interventions for advanced Parkinson's disease (see full guideline chapter 9).

M.1 Call for evidence as issued

The call for evidence was issued on 22 June 2015. It is reproduced below.

Call for evidence to all registered stakeholders

Updated NICE guidance on the diagnosis and management of Parkinson's disease in adults

Dear Stakeholder

The National Institute for Health and Care Excellence (NICE) has commissioned its Internal Clinical Guidelines team (ICG) to update guidance on the diagnosis and management of Parkinson's disease in adults.

Your organisation is invited to submit data that meets the requirements set out below to assist the guideline development process.

We have already carried out extensive searches to cover published clinical literature. Upon reviewing the literature, we have reason to believe there may be relevant evidence in addition to that identified by the searches that is not yet available in published form.

We are evaluating the following review question:

What is the comparative clinical effectiveness and cost effectiveness, for people with Parkinson's disease whose symptoms are no longer controlled by existing pharmacological treatment(s), of:

- **deep brain stimulation surgery (of any surgical target) or**
- **levodopa and carbidopa intestinal gel or**
- **best medical treatment (which may or may not include apomorphine infusion).**

We are interested in data covering the following areas:

1. Unpublished data from any randomised controlled trial comparing 2 or more of the above interventions
2. Unpublished non-randomised data detailing long-term follow-up (1 year or longer) of any of the above interventions (to inform our original health economic analysis)
3. Existing health economic data or models comparing any 2 or more of the above interventions.

We require data reporting any of the following outcomes:

- Patient-reported outcomes:
  - EQ-5D (summary index score)
  - PDQ-39 (at domain and summary level)
  - PDQ-8 (at domain and summary level)
  - ON and/or OFF times (reported as hours per day or quartile percentages)
- Markers of disease progression (in ON and/or OFF states):
  - UPDRS (total score and/or subscores for parts III, IVa IVb and IVc)

- Hoehn and Yahr stages
- Resource use (including concomitant medication and care requirements)
- Rates of entry to full-time institutional care (e.g. time-to-event data)
- Any adverse events (rates or numbers of events and people)

Data can be submitted in aggregate (with appropriate measures of dispersion) or appropriately anonymised individual form. In addition to the outcomes listed, supporting data covering the number of participants, baseline participant characteristics and any concomitant medication(s) taken should be submitted.

Data should be submitted in Microsoft Word, Microsoft Excel, Adobe PDF or comma-separated value formats. Health economic models may also be submitted as R, WinBUGS or TreeAge files. If you wish to submit data in any other format, please get in touch to discuss with us.

You should ensure that any data you wish to submit:

- Directly addresses the review question listed
- Fits into 1 of the 3 categories of data required
- Reports outcomes listed and supporting data
- Are in the format(s) listed.

There is no need to submit data that have already been published.

Please note that all evidence submitted will be reviewed against the inclusion criteria indicated here. Only evidence that is eligible will be presented to the Guideline Development Group for consideration. All such evidence will be subject to the same process of critical appraisal that applies to published evidence identified in our reviews. We are in no way bound to use submitted data.

Please read the instructions below to identify the types of information that can be accepted. A full description of NICE's guideline development process and guidance of stakeholders is available from the NICE website.

If you wish to make a submission please email it to stephanie.mills@nice.org.uk. Alternatively, submissions can be posted to Stephanie Mills, National Institute for Health and Care Excellence, Level 1A City Tower, Piccadilly Plaza, Manchester M1 4BD. Submissions should arrive by **12 noon on Monday 20 July 2015.**

Please acknowledge receipt of this letter by email, indicating whether your organisation will be submitting evidence for consideration

Yours sincerely

Stephanie Mills

Project Manager

M.2 Responses to call for evidence

A total of 10 stakeholders and other data-holders made submissions in response to the call for evidence. These were considered against the eligibility criteria for the review questions and the additional criteria specified in the call for evidence. Brief details of the submitted evidence are tabulated below. Evidence that was used to inform GDG decision making is described in greater detail the full guideline. Evidence that was excluded is noted with reasons below.

Contributor	Description	Include / exclude
AbbVie	Poster retrospective review of LCIG AEs and discontinuations	EXCLUDE: poster only; no comparative data; likely overlap in participants with other retrospective LCIG evidence used in health economic model
Boston Scientific	2 poster presentations from nonrandomised cases series assessing new type of DBS stimulator	EXCLUDE: posters only; no comparative data; no extended follow-up
Britannia	Poster and draft paper on using subcutaneous apomorphine to reduce morning response delay	EXCLUDE: not an outcome listed in call for evidence or review protocol; no extended follow-up
	Draft paper on real-world resource use with apomorphine	EXCLUDE: non-comparative cost data
Global Kinetics	3 case studies of device for measuring dyskinesia	EXCLUDE: no interventions or outcomes of interest
King's College Hospital NHS Foundation Trust	Various published papers	EXCLUDE: all relevant publications considered in systematic searches
	Patient-level data from EuroInf observational study (6-month follow-up)	Considered against data requirements of original health economic model, but EXCLUDED as more robust sources of health-related quality of life data, with longer follow-up were available
Medtronic	Unpublished data from EARLYSTIM RCT (intermediate follow-up points and estimated EQ-5D)	EXCLUDE: not advanced Parkinson's
	Unpublished comparison of EARLYSTIM and US Veterans Affairs data	EXCLUDE: not advanced Parkinson's
	Unpublished abstract detailing long-term follow-up of the EARLYSTIM pilot	EXCLUDE: non-comparative data; not advanced Parkinson's
	Unpublished evidence on safety and performance of Medtronic DBS devices as analysed from the Medtronic Implantable Systems Performance Registry	Considered against data requirements of original health economic model, but EXCLUDED as alternative sources of safety data with associated costs were available (PDSURG)
	Draft cost–utility model EARLYSTIM data: DBS compared with BMT for patients with early complications of PD in France	Does not formally meet criteria for call for evidence (not advanced Parkinson's) but INCLUDE for early DBS question
	Outline of planned cost–utility model using EARLYSTIM to compare DBS with BMT for patients with early complications of PD in UK	EXCLUDE as incomplete at time of submission; subsequently published (Fundament et al., 2016) and included in early DBS question

Contributor	Description	Include / exclude
	3 abstracts detailing adaptations of existing analyses for different jurisdictions (none Sweden, France, USA)	EXCLUDE: abstracts only and model being adapted (Eggington et al. 2013) is included evidence
	Draft paper estimating UK drug costs based on observed patient drug use in the EARLYSTIM clinical trial	EXCLUDE: not advanced Parkinson's
	Poster giving cost comparison of DBS -v- subcutaneous apomorphine in advanced Parkinson's	EXCLUDE: poster only; costs only
	Proposal for UK data linkage study to give costs by disease stage	EXCLUDE: proposal only
PDSURG	Draft of economic evaluation paper	INCLUDE (paper subsequently published [Macintosh et al. 2016])
	Patient-level data	INCLUDE (used to estimate population-specific treatment effects; various other data used in original health economic model as detailed in methods)
University College London	Poster on impulsive compulsive behaviours in patients with Parkinson's disease treated with apomorphine, with underpinning patient-level data	EXCLUDE: not an outcome listed in call for evidence or review protocol; no extended follow-up
University of Marburg	Draft cost–utility model assessing early DBS	Does not formally meet criteria for call for evidence (not advanced Parkinson's) but INCLUDE for early DBS question