

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Technology appraisals

Patient access scheme submission template

October 2009

1 Introduction

The 2009 Pharmaceutical Price Regulation Scheme (PPRS) (www.dh.gov.uk/en/Healthcare/Medicinespharmacyandindustry/Pharmaceuticalalpriceregulationscheme/2009PPRS) is a non-contractual scheme between the Department of Health and the Association of the British Pharmaceutical Industry. The purpose of the 2009 PPRS is to ensure that safe and cost-effective medicines are available on reasonable terms to the NHS in England and Wales. One of the features of the 2009 PPRS is to improve patients' access to medicines at prices that better reflect their value through patient access schemes.

Patient access schemes are arrangements which may be used on an exceptional basis for the acquisition of medicines for the NHS in England and Wales. Patient access schemes propose either a discount or rebate that may be linked to the number, type or response of patients, or a change in the list price of a medicine linked to the collection of new evidence (outcomes). These schemes help to improve the cost effectiveness of a medicine and therefore allow the National Institute for Health and Clinical Excellence (NICE) to recommend treatments which it would otherwise not have found to be cost effective. More information on the framework for patient access schemes is provided in the 2009 PPRS (www.dh.gov.uk/en/Healthcare/Medicinespharmacyandindustry/Pharmaceuticalalpriceregulationscheme/2009PPRS).

Patient access schemes are proposed by a pharmaceutical company and agreed with the Department of Health, with input from the Patient Access Schemes Liaison Unit (PASLU) within the Centre for Health Technology Evaluation at NICE.

2 Instructions for manufacturers and sponsors

This document is the patient access scheme submission template for technology appraisals. If manufacturers and sponsors want the National Institute for Health and Clinical Excellence (NICE) to consider a patient access scheme as part of a technology appraisal, they should use this template. NICE can only consider a patient access scheme after formal referral from the Department of Health.

The template contains the information NICE requires to assess the impact of a patient access scheme on the clinical and cost effectiveness of a technology, in the context of a technology appraisal, and explains the way in which background information (evidence) should be presented. If you are unable to follow this format, you must state your reasons clearly. You should insert 'N/A' against sections that you do not consider relevant, and give a reason for this response.

Please refer to the following documents when completing the template:

- 'Guide to the methods of technology appraisal'
(www.nice.org.uk/aboutnice/howwework/devnicetech/technologyappraisalprocessguides/guidetothemethodsoftechnologyappraisal.jsp)
- 'Specification for manufacturer/sponsor submission of evidence'
(<http://www.nice.org.uk/aboutnice/howwework/devnicetech/singletechnologypappraisalsubmissiontemplates.jsp>) and
- Pharmaceutical Price Regulation Scheme 2009
(www.dh.gov.uk/en/Healthcare/Medicinespharmacyandindustry/Pharmaceuticalpriceregulationscheme/2009PPRS).

For further details on the technology appraisal process, please see NICE's 'Guide to the single technology appraisal (STA) process' and 'Guide to the multiple technology appraisal (MTA) process'
(http://www.nice.org.uk/aboutnice/howwework/devnicetech/technologyappraisalprocessguides/technology_appraisal_process_guides.jsp). The

'Specification for manufacturer/sponsor submission of evidence' provides details on disclosure of information and equality issues.

Make the submission as brief and informative as possible. Only mark information as confidential when absolutely necessary. Sufficient information must be publicly available for stakeholders to comment on the full content of the technology appraisal, including details of the proposed patient access scheme. Send submissions electronically to NICE in Word or a compatible format, not as a PDF file.

Appendices may be used to include additional information that is considered relevant to the submission. Do not include information in the appendices that has been requested in the template. Appendices should be clearly referenced in the main submission.

When making a patient access scheme submission, include:

- an updated version of the checklist of confidential information, if necessary
- an economic model with the patient access scheme incorporated, in accordance with the 'Guide to the methods of technology appraisal' (www.nice.org.uk/aboutnice/howwework/devnicetech/technologyappraisalprocessguides/guidetothemethodsoftechnologyappraisal.jsp).

If you are submitting the patient access scheme at the end of the appraisal process, you should update the economic model to reflect the assumptions that the Appraisal Committee considered to be most plausible. No other changes should be made to the model.

3 Details of the patient access scheme

3.1 Please give the name of the technology and the disease area to which the patient access scheme applies.

Golimumab (brand name: Simponi). The patient access scheme, as approved by the Department of Health, applies to all current licensed indications; rheumatoid arthritis (RA), psoriatic arthritis (PsA), ankylosing spondylitis (AS) and any future indications.

3.2 Please outline the rationale for developing the patient access scheme.

The Scheme functions in such a way as to avoid any additional administrative burden or cost over and above that needed to provide a patient with the 50 mg dose. It thus removes any concerns that prescribing patients over 100 kg a 100 mg (2x 50 mg auto injector) dose will increase costs to the NHS in England and Wales.

3.3 Please describe the type of patient access scheme, as defined by the PPRS.

The PAS is financially based and has been designed to have a minimal impact on both administration levels and clinician workloads within the NHS setting. The PAS provides equal opportunities to eligible patients irrespective of response levels.

3.4 Please provide specific details of the patient population to which the patient access scheme applies. Does the scheme apply to the whole licensed population or only to a specific subgroup (for example, type of tumour, location of tumour)? If so:

- How is the subgroup defined?

- If certain criteria have been used to select patients, why have these have been chosen?
- How are the criteria measured and why have the measures been chosen?

The Scheme applies only to those NHS patients who weigh more than 100 kg and whose disease does not show an adequate clinical response after three or four doses of Golimumab.

The patient's weight will be determined by the treating clinician, or a member of their team, using an appropriate (as determined by the hospital or clinic) measurement device. Treating clinicians may recommend increasing the dose of golimumab to 100 mg after three or four doses of 50 mg. In order for the NHS trust or primary care trust to receive the 100 mg dose (two 50 mg auto injectors or pre-filled syringes) at the same price as the 50 mg dose, they must tick the 100 mg box on the homecare prescription form.

There will be no patients from this subgroup who will be excluded from the scheme.

3.5 Please provide details of when the scheme will apply to the population specified in 3.4. Is the scheme dependent on certain criteria, for example, degree of response, response by a certain time point, number of injections? If so:

- Why have the criteria been chosen?
- How are the criteria measured and why have the measures been chosen.

Please refer to 3.4

3.6 What proportion of the patient population (specified in 3.4) is expected to meet the scheme criteria (specified in 3.5)?

Not applicable

3.7 Please explain in detail the financial aspects of the scheme. How will any rebates be calculated and paid?

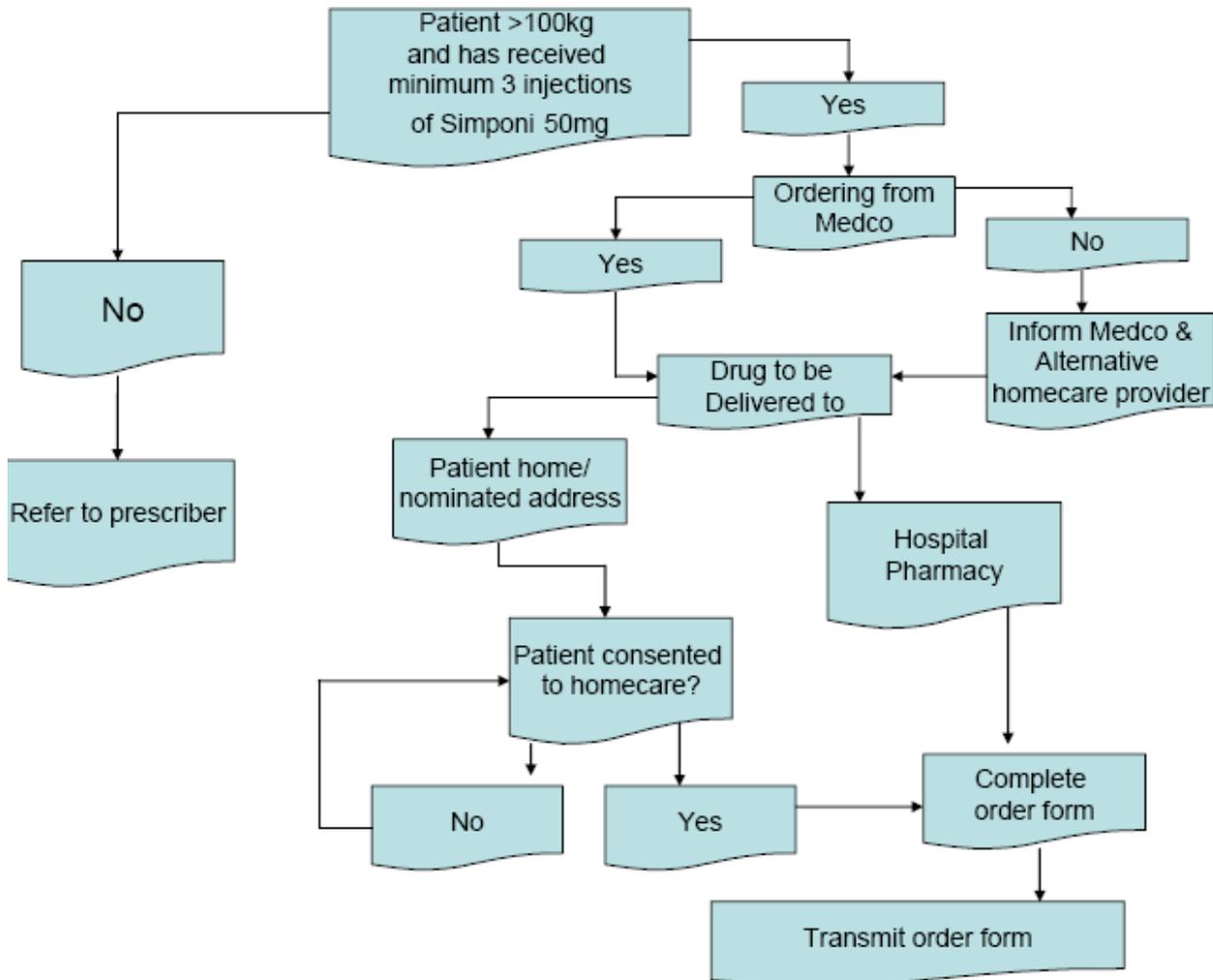
Rebates will generally not be provided through this patient access scheme; the discount will only be provided to the NHS when golimumab is ordered. MSD believes that the scheme cannot be simplified further.

3.8 Please provide details of how the scheme will be administered. Please specify whether any additional information will need to be collected, explaining when this will be done and by whom.

It is expected that over 90% of patients would receive golimumab through a homecare provider. However, a small proportion of patients may receive golimumab while in hospital or as hospital outpatients. If patients start golimumab treatment in hospital, the manufacturer expects that these patients would be discharged and transferred to homecare during the period of receiving the first three or four doses of 50 mg. Therefore the majority of patients weighing over 100 kg would be receiving golimumab through a homecare provider before their treating clinicians make the decision to increase their dose. However, in the event that a patient is prescribed the increased dose, and receives the dose within the hospital setting, the patient access scheme would still be available. MSD will work with the individual NHS trust to minimise any administrative burden that may arise.

The wholesaler, Medco Health Solutions Ltd, will provide MSD with aggregate data, but these data will not include information on patients or clinicians. These data will be used to determine the amount of golimumab prescribed to patients within the scheme at the 100 mg dose.

3.9 Please provide a flow diagram that clearly shows how the scheme will operate. Any funding flows must be clearly demonstrated.



3.10 Please provide details of the duration of the scheme.

The scheme will be effective until NICE reviews the guidance for golimumab for the current indications (RA, PsA and AS) and any future indications, subject to agreement with the Department of Health. MSD does not currently consider there to be any circumstances under which it would terminate the proposed scheme.

3.11 Are there any equity or equalities issues relating to the scheme, taking into account current legislation and, if applicable, any

concerns identified during the course of the appraisal? If so, how have these been addressed?

There are no equity or equalities issues relating to the scheme.

- 3.12 If available, please list any scheme agreement forms, patient registration forms, pharmacy claim forms/rebate forms, guides for pharmacists and physicians and patient information documents. Please include copies in the appendices.

Attached in the appendices are the Medco service level agreement and the guide for NHS professionals.

- 3.13 In the exceptional case that you are submitting an outcome-based scheme, as defined by the PPRS, please also refer to appendix B.

Not applicable

Cost effectiveness

- 3.14 If the population to whom the scheme applies (as described in sections 3.4 and 3.5) has not been presented in the main manufacturer/sponsor submission of evidence for the technology appraisal (for example, the population is different as there has been a change in clinical outcomes or a new continuation rule), please (re-)submit the relevant sections from the 'Specification for manufacturer/sponsor submission of evidence' (particularly sections 5.5, 6.7 and 6.9). You should complete those sections both with and without the patient access scheme. You must also complete the rest of this template.

The scheme was developed to ensure that in the unlikely event prescribing of the 100mg occurred there would be no additional financial burden for the NHS.

Given the predicted rarity of the event and that the additional dose carries no additional cost to the NHS, MSD has not included the PAS within the cost-effectiveness analyses.

The estimated maximum number of patients expected to receive Golimumab at a dose of 100mg (based on current incidence, prevalence and market share) are as follows:

Calculation of expected number of patients over 100 kg receiving golimumab at a dose of 100 mg

Year	Rheumatoid arthritis	Ankylosing spondylitis	Psoriatic arthritis	Total expected patient numbers per year
Year 1	14	15	2	31
Year 2	32	36	5	73
Year 3	65	63	8	136
Year 4	111	94	12	217

This can be seen to be a very small number of patients when compared to the total number of patients eligible for treatment with biologics as displayed below.

Total number of patients eligible for treatment with Biologics

Year	Rheumatoid arthritis	Ankylosing spondylitis	Psoriatic arthritis	Total expected patient numbers per year
Year 1	49,121	17,775	2,225	69,121
Year 2	49,492	17,900	2,241	69,633
Year 3	49,846	18,026	2,256	70,128
Year 4	50,186	18,153	2,272	70,611

The PAS will absorb any additional cost should dose escalation occur. However, given that there is no direct clinical data supporting dose escalation for patients weighing >100kg, MSD will not be advocating dose escalation and will not be marketing golimumab with dose escalation in mind.

3.15 If you are submitting the patient access scheme at the end of the technology appraisal process, you should update the economic model to reflect the assumptions that the Appraisal Committee considered to be most plausible. No other changes should be made to the model.

[Please refer to 3.14](#)

3.16 Please provide details of how the patient access scheme has been incorporated into the economic model. If applicable, please also provide details of any changes made to the model to reflect the assumptions that the Appraisal Committee considered most plausible.

[Please refer to 3.14](#)

3.17 Please provide the clinical effectiveness data resulting from the evidence synthesis and used in the economic model which includes the patient access scheme.

[Please refer to 3.14](#)

3.18 Please list any costs associated with the implementation and operation of the patient access scheme (for example, additional pharmacy time for stock management or rebate calculations). A suggested format is presented in table 1. Please give the reference source of these costs. Please refer to section 6.5 of the 'Specification for manufacturer/sponsor submission of evidence'.

[The scheme would not require any additional tests, interventions or appointments with healthcare professionals. Both the 50 mg and 100 mg doses of golimumab are administered by subcutaneous injection. MSD does not expect any additional treatment-related cost to the NHS when a patient is prescribed the 100 mg dose compared to the 50 mg dose.](#)

Where golimumab at the 100 mg dose is delivered through homecare arrangements there will be minimal administration burden created by the patient access scheme. The patient access scheme is clinically plausible because clinicians already complete homecare-related paperwork for existing therapies and this scheme only requires a tick in a different box to prescribe the 100 mg dose for patients weighing over 100 kg.

- 3.19 Please provide details of any additional treatment-related costs incurred by implementing the patient access scheme. A suggested format is presented in table 2. The costs should be provided for the intervention both with and without the patient access scheme. Please give the reference source of these costs.

[Please refer to 3.18](#)

Summary results

Base-case analysis

- 3.20 Please present in separate tables the cost-effectiveness results as follows.¹
- the results for the intervention without the patient access scheme
 - the results for the intervention with the patient access scheme.

A suggested format is shown below (table 3).

[Please refer to 3.14](#)

¹ For outcome-based schemes, please see section 5.2.8 in appendix B.

3.21 Please present in separate tables the incremental results as follows.²

- the results for the intervention without the patient access scheme
- the results for the intervention with the patient access scheme.

List the interventions and comparator(s) from least to most expensive. Present the incremental cost-effectiveness ratios (ICERs) in comparison with baseline (usually standard care), and the incremental analysis ranking technologies in terms of dominance and extended dominance. A suggested format is presented in table 4.

[Please refer to 3.14](#)

Sensitivity analyses

3.22 Please present deterministic sensitivity analysis results as described for the main manufacturer/sponsor submission of evidence for the technology appraisal. Consider using tornado diagrams.

[Please refer to 3.14](#)

3.23 Please present any probabilistic sensitivity analysis results, and include scatter plots and cost-effectiveness acceptability curves.

[Please refer to 3.14](#)

² For outcome-based schemes, please see section 5.2.9 in appendix B.

3.24 Please present scenario analysis results as described for the main manufacturer/sponsor submission of evidence for the technology appraisal.

[Please refer to 3.14](#)

3.25 If any of the criteria on which the patient access scheme depends are clinical variable (for example, choice of response measure, level of response, duration of treatment), sensitivity analyses around the individual criteria should be provided, so that the Appraisal Committee can determine which criteria are the most appropriate to use.

[Please refer to 3.14](#)

Impact of patient access scheme on ICERs

3.26 For financially based schemes, please present the results showing the impact of the patient access scheme on the ICERs for the base-case and any scenario analyses. A suggested format is shown below (see table 5). If you are submitting the patient access scheme at the end of the appraisal process, you must include the scenario with the assumptions that the Appraisal Committee considered to be most plausible.

[Please refer to 3.14](#)

4 Appendices

4.1 *Appendix A: Additional documents*

4.1.1 If available, please include copies of patient access scheme agreement forms, patient registration forms, pharmacy claim forms/rebate forms, guides for pharmacists and physicians, patient information documents.

4.1.2 Simponi Patient Access Scheme How To Guide

4.1.3 Medco Service Level Agreement

4.2 Appendix B: Details of outcome-based schemes

4.2.1 If you are submitting a proven value: price increase scheme, as defined in the PPRS, please provide the following information:

- the current price of the intervention
- the proposed higher price of the intervention, which will be supported by the collection of new evidence
- a suggested date for when NICE should consider the additional evidence.

Not Applicable

4.2.2 If you are submitting an expected value: rebate scheme, as defined in the PPRS, please provide the following details:

- the current price of the intervention (the price that will be supported by the collection of new evidence)
- the planned lower price of the intervention in the event that the additional evidence does not support the current price
- a suggested date for when NICE should consider the additional evidence.

Not Applicable

4.2.3 If you are submitting a risk-sharing scheme, as defined in the PPRS, please provide the following details:

- the current price of the intervention (the price that will be supported by the collection of new evidence)
- the proposed relationship between future price changes and the evidence to be collected.

Not Applicable

4.2.4 For outcome-based schemes, as defined in the PPRS, please provide the full details of the new information (evidence) planned to be collected, who will collect it and who will carry the cost associated with this planned data collection. Details of the new information (evidence) may include:

- design of the new study
- patient population of the new study
- outcomes of the new study
- expected duration of data collection
- planned statistical analysis, definition of study groups and reporting (including uncertainty)
- expected results of the new study
- planned evidence synthesis/pooling of data (if applicable)
- expected results of the evidence synthesis/pooling of data (if applicable).

Not Applicable

4.2.5 If you are submitting a risk-sharing scheme, please specify the period between the time points when the additional evidence will be considered.

Not Applicable

4.2.6 Please provide the clinical effectiveness data resulting from the evidence synthesis and used in the economic modelling of the patient access scheme at the different time points when the additional evidence is to be considered.

Not Applicable

4.2.7 Please provide the other data used in the economic modelling of the patient access scheme at the different time points when the additional evidence is to be considered. These data could

include cost/resource use, health-related quality of life and utilities.

Not Applicable

4.2.8 Please present the cost-effectiveness results as follows.

- For proven value: price increase schemes, please summarise in separate tables:
 - the results based on current evidence and current price
 - the anticipated results based on the expected new evidence and the proposed higher price.
- For expected value: rebate schemes, please summarise in separate tables:
 - the results based on the expected new evidence and the current price (which will be supported by the additional evidence collection)
 - the results based on the current evidence and the lower price (if the new evidence is not forthcoming).
- For risk-sharing schemes, please summarise in separate tables:
 - the results based on current evidence and current price
 - the results based on the expected new evidence and the current price (which will be supported by the additional evidence collection)
 - the results based on the current evidence and the lower price (if the new evidence is not forthcoming)
 - the anticipated results based on the expected new evidence and the proposed higher price.

A suggested format is shown in table 3, section 4.7.

Not Applicable

4.2.9 Please present in separate tables the incremental results for the different scenarios as described above in section 5.2.8 for the type of outcome-based scheme being submitted.

List the interventions and comparator(s) from least to most expensive. Present the incremental cost-effectiveness ratios (ICERs) in comparison with baseline (usually standard care), and the incremental analysis ranking technologies in terms of dominance and extended dominance. A suggested format is presented in table 4, section 4.8.

[Not Applicable](#)