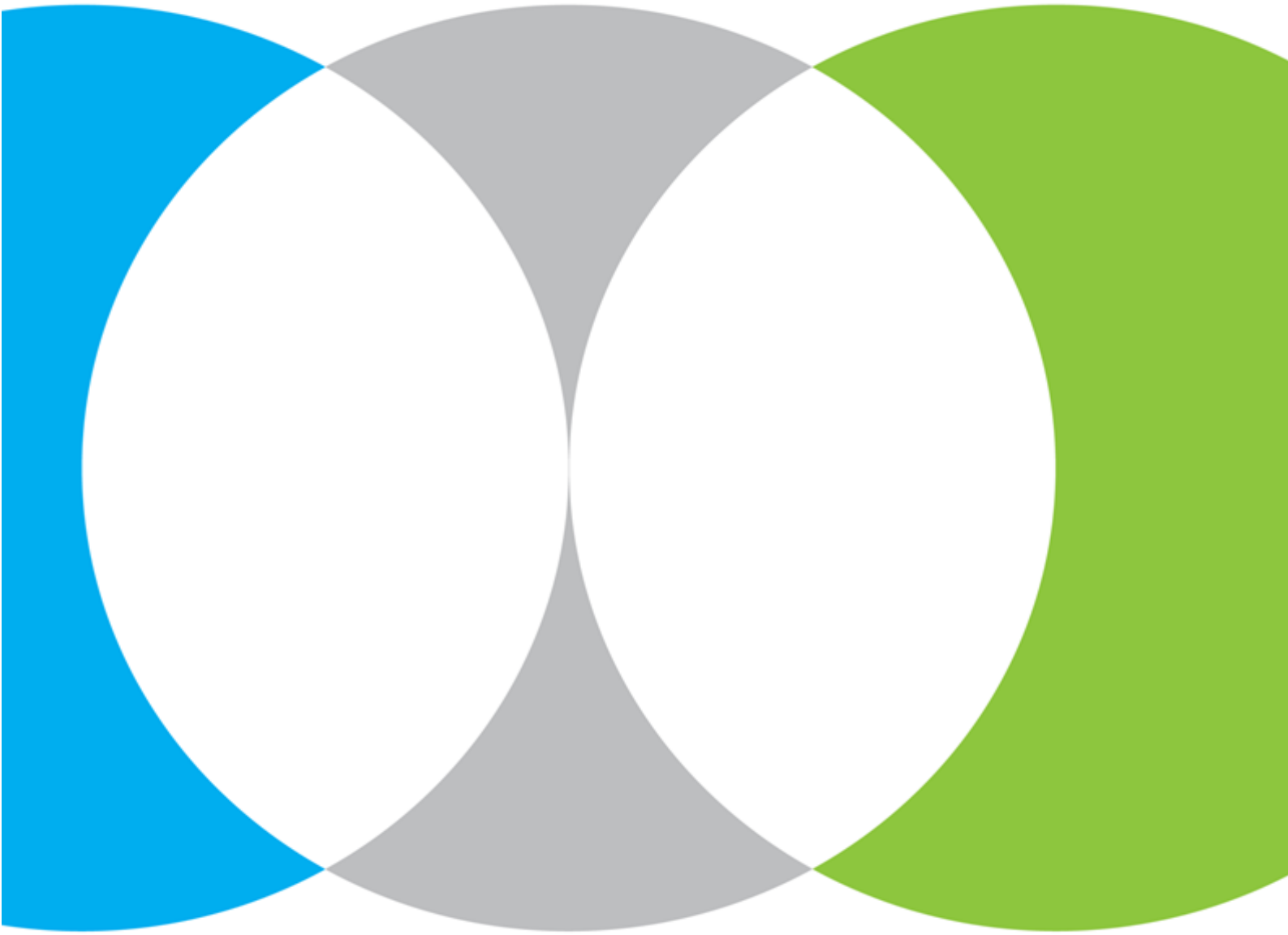
Guidance

UK PharmaScan



UK PharmaScan

provided by NICE on behalf of abpi, DH Department of Health, Llywodraeth Cymru Welsh Assembly Government, The Scottish Government, Northern Ireland Executive

1. Introduction

1.1 About this guidance

This guidance is for pharmaceutical companies. All descriptions and screenshots are accurate at date of production but may differ slightly from what you see when using the live service.

1.2 About *UK PharmaScan*

UK PharmaScan has been designed to help the NHS better plan for the introduction of new medicines, indications, licence extensions and formulations, in order to support their faster adoption by the NHS.

The time devoted to horizon scanning activities by your company should be reduced over time by not having to give the same factual information to each of the 7 horizon scanning organisations in the UK and will enable you to re-invest that time on more fruitful and meaningful dialogue about interpretation of that information.

UK PharmaScan should contain data on all of your new medicines and biosimilars, new indications / licence extensions, new formulations for existing medicines and in-licensed medicines developed by a third party, which are in phase III or three years from estimated launch, whichever is the earliest. Please enter those medicines closest to launch first.

As there can be multiple indications and licensing applications for a single drug, information is held in the database in the form of two separate but linked records:

- A **drug record** which holds basic information about the name of the drug
- A **technology record** which holds the information on the indication, licence extension and/or formulation which is subject to the individual licensing application.

1.3 Further information and help

Further information can be found on the *UK PharmaScan* website. The About Us section provides answers to commonly asked questions and includes links to the *UK PharmaScan* flyer and slide deck.

For logged in users, the Resources section of the website houses links to supporting resources including frequently asked questions, skeleton Standard Operating Procedure, Examples of Ideal Records, Case Studies, User Bulletins and Top Tips.

The *UK PharmaScan* enquiries team is based at the National Institute for Health and Care Excellence (NICE) and is available to users from 9am to 5pm Monday to Friday to assist with any access and/or technical issues. Please report all technical issues to the enquiries team so we can learn from your experience and ensure issues are addressed. The team can be contacted on 0300 323 0159 or by email at contactus@ukpharmascan.org.uk.

2 Registration

2.1. Registering your company and assigning a champion user

At registration stage all pharmaceutical companies are required to select an appropriate person to be the champion user.

The champion user is the senior user within a company responsible for registering that company with *UK PharmaScan* and has the authority to review, approve and maintain access permissions to other users (standard users) in that company.

The champion user is responsible for:

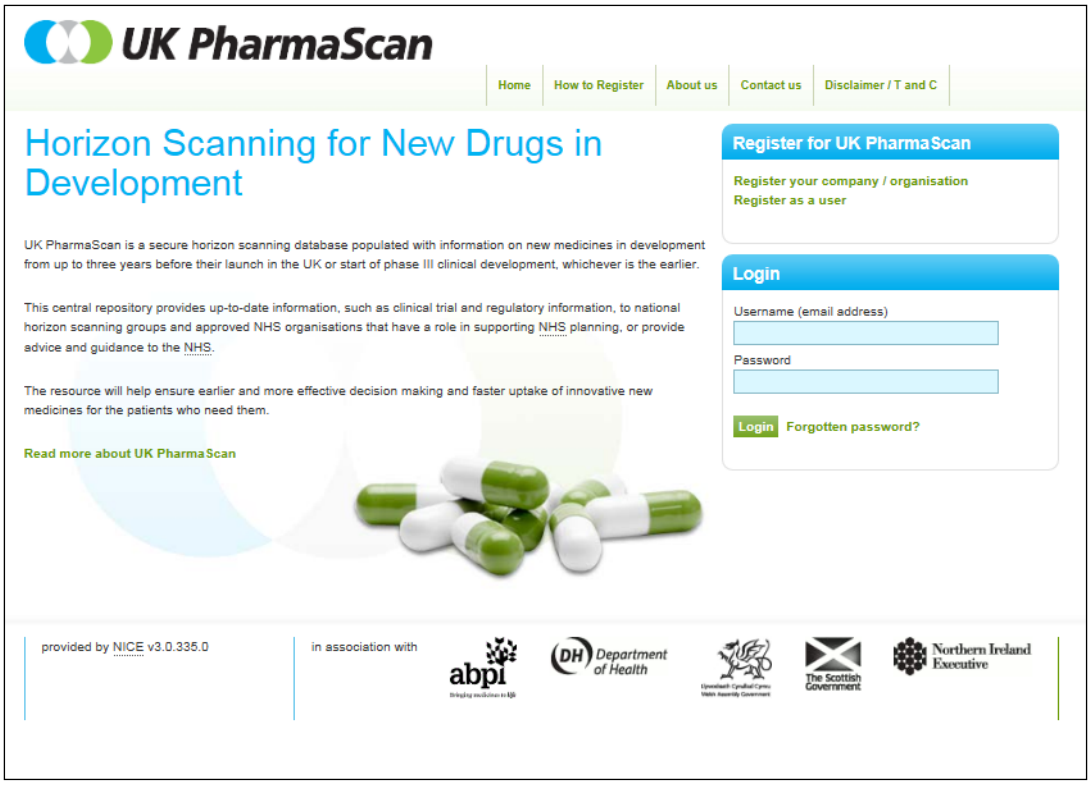
- deactivating users immediately they no longer require access, for example, moving departments or leaving the company.
- ensuring that a new champion user is assigned when they are on annual leave/extended leave or when they leave the company.

A maximum of 5 active users including the champion user are allowed at any one time.

If you are not your company's champion user, please go to section 2.3.
Please note, all pharmaceutical companies should register on *UK PharmaScan*, including those which have no products to enter into the database at the time of registration.

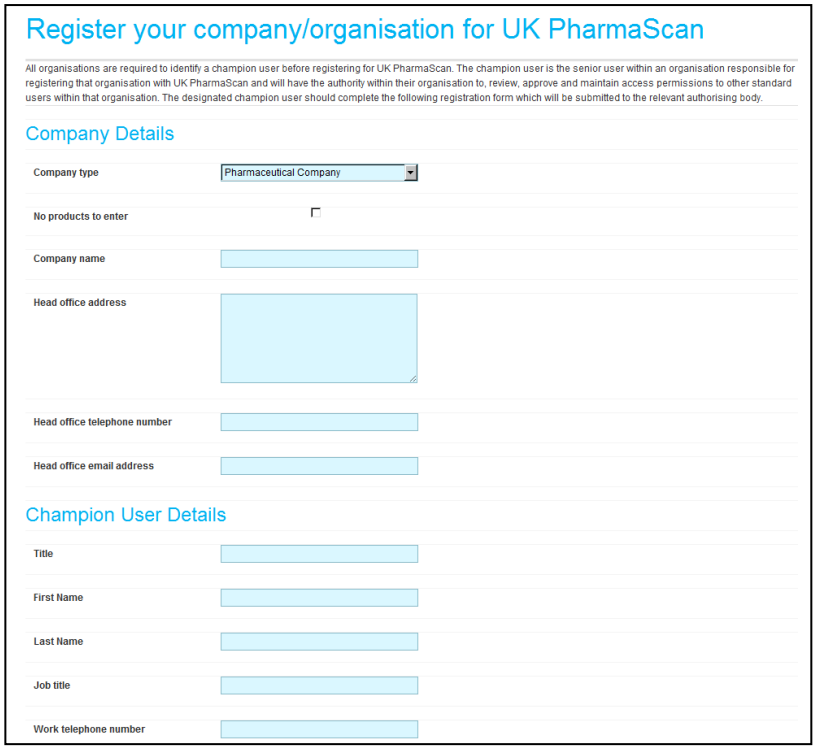
Please check your company is not already registered by checking the drop down list of companies available at:
https://www.ukpharmascan.org.uk/userdetails/register.

- ✓ To register your company, go to the *UK PharmaScan* website homepage at: www.ukpharmascan.org.uk



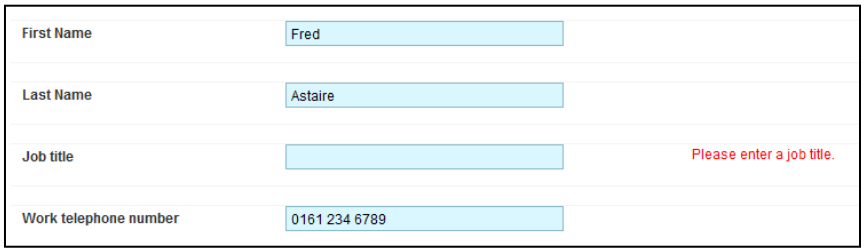
✓ Click **Register your company / organisation** in the box on the right of the page

✓ Complete all the fields on the form



- ✓ If you do not have any products to enter into the database, tick the **No products to enter** box

All fields are mandatory. Fields which have not been completed will be highlighted.

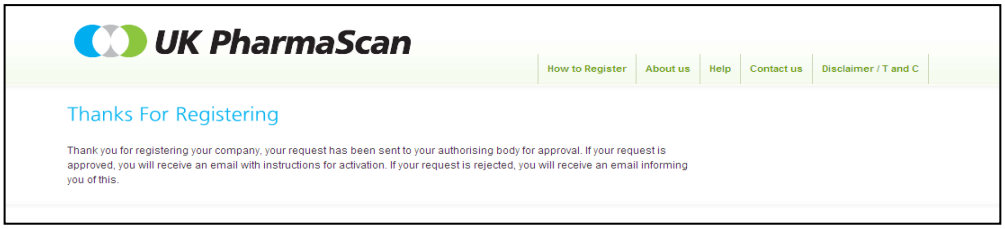


Your password should contain at least one number and one uppercase letter, and be up to 15 characters long.

- ✓ Click the **Register** button

After you have submitted the registration form, the *UK PharmaScan* authorising body for pharmaceutical companies, the Association of the British Pharmaceutical Industry (ABPI) will be asked to approve the proposed company and champion user. This process takes two to three working days.

You will see a page telling you that your request has been sent for approval.



For instructions on how to activate your account, go to section 2.4.

2.2. Data Inputter agreement

If your application is successful, you will receive an activation email (see section 2.4) and an email from ABPI sent on behalf of the Department of Health including a copy of the Data Inputter agreement. The champion user is asked to print 2 copies to be signed by a senior person in the company with the authority to act on behalf of and to bind the company to the terms of the agreement.

The Data Inputter agreement is a legal agreement between the Secretary of State for Health acting through the Department of Health and your company. The agreement includes details on:

- Registration and provision of the database
- Data and proprietary rights
- Conditions of use
- Intellectual property rights acknowledgement
- Representations and warranties
- Limitation of liability
- Freedom of Information Act
- Data protection and confidential information

One signed copy should be retained for your records, the second copy should be sent to NICE – see agreement for full address.

2.3. Standard users

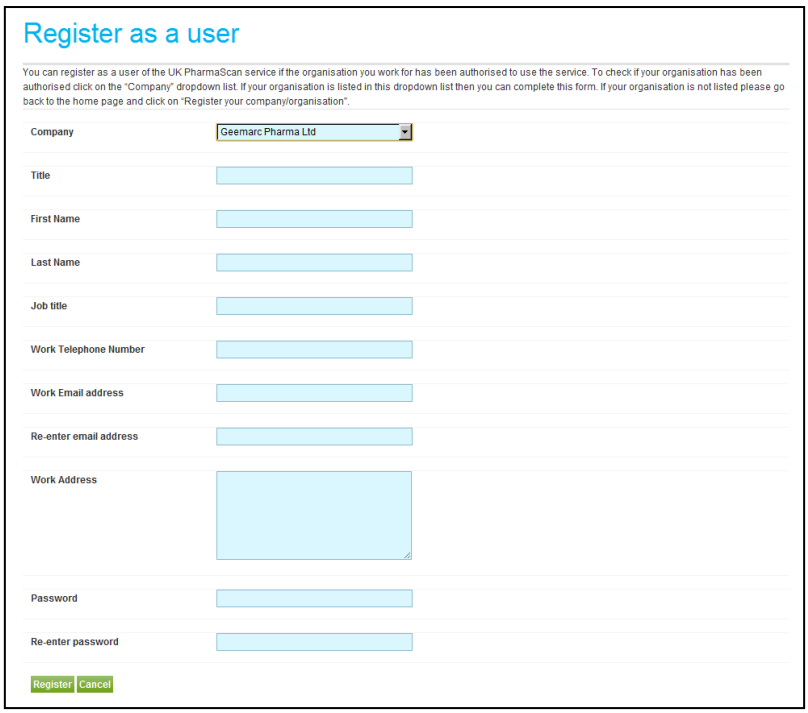
Once a champion user has activated their account, other members of the same company can apply for access to *UK PharmaScan* as standard users.

- ✓ Go to the *UK PharmaScan* website homepage: www.ukpharmascan.org.uk
- ✓ Click **Register as a user** in the box on the right of the page

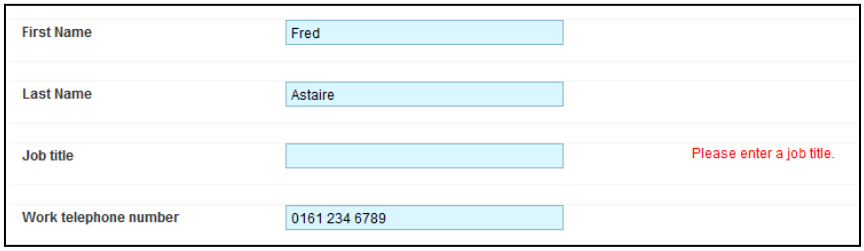
✓ Select your company from the **Company** dropdown list

If your company is not listed, it has not yet been approved and/or the champion user has not activated their account. You will need to wait until the champion user has activated their account before you can register as a standard user.

✓ Complete all the fields on the form



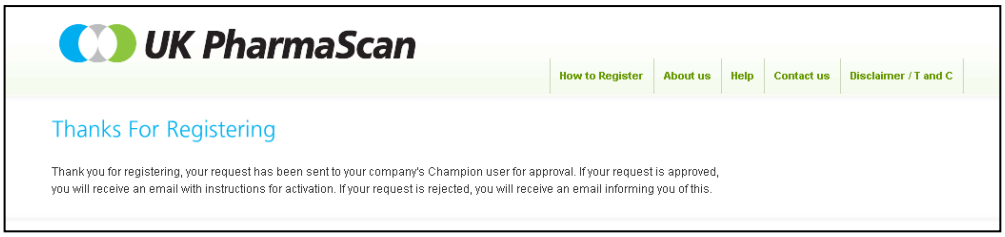
All fields are mandatory. You will be asked to complete any uncompleted fields.



Your **password** should contain at least one number and one uppercase letter, and be up to 15 characters long.

- ✓ Click the **Register as a user** button

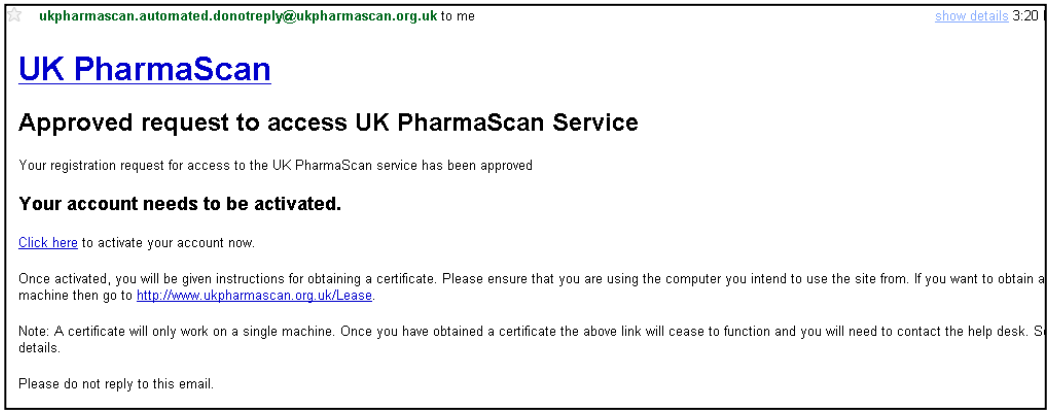
You will see a page advising you that your request has been sent to your champion user for approval. Please remember your password as you will need this for accessing the database once your application has been approved.



Your request will automatically be submitted to your company's champion user, who is responsible for reviewing your application.

2.4. Activation

If your application is successful, you will receive an activation email.



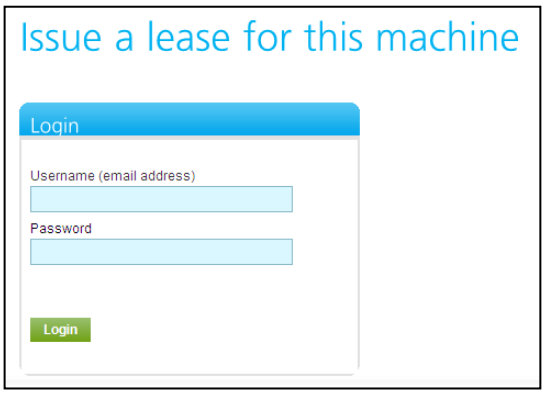
The email contains a link to activate your account. As part of the activation process you will need to download a lease onto your PC. This lease is used to verify that you are an approved user of *UK PharmaScan* when you login to the site in future.

A lease will only work on a single PC and each user can only be issued with 1 lease, so you should ensure that you are using the PC on which you intend to use *UK PharmaScan*. If you do not have Flash installed, you should also ensure that you are using your usual browser.

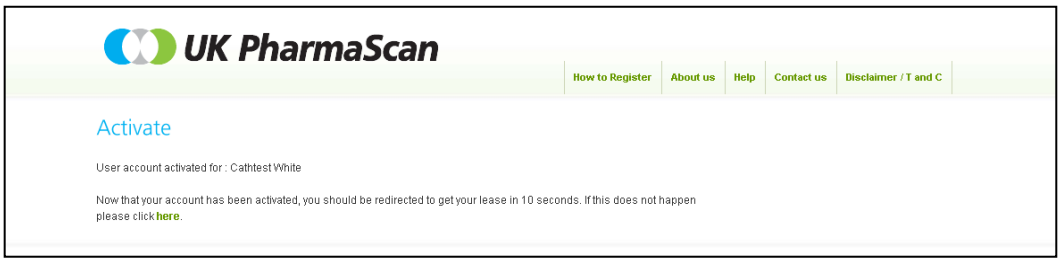
- ✓ Click on the activation link in the email

The activation link will take you to a page on the *UK PharmaScan* site where you can download your lease.

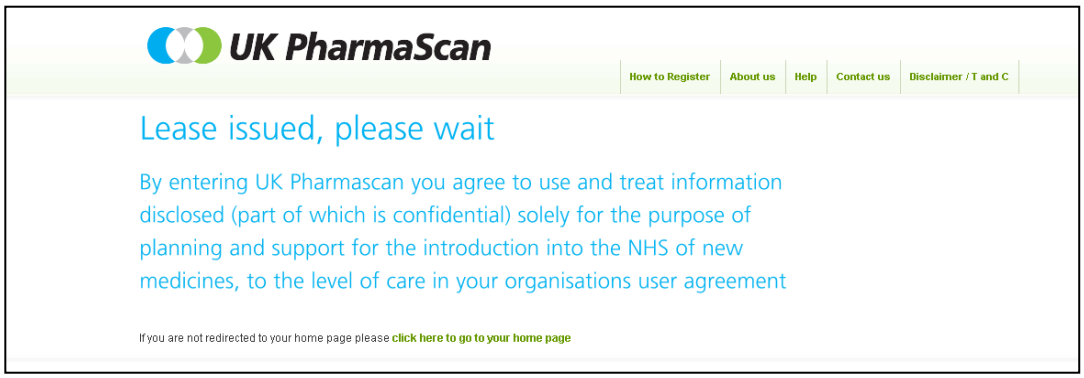
- ✓ Enter your details in the **Username (email address)** and **Password** fields
- ✓ Click the **Login** button



You will receive confirmation that your details have been accepted and you will be automatically redirected to download your lease.



Once your lease has been downloaded you will be presented with a confirmation message.



You will then be automatically redirected to your own homepage (section 4).

If you have any problems activating your account or downloading the lease, please contact the *UK PharmaScan* enquiries team (contactus@ukpharmascan.org.uk).

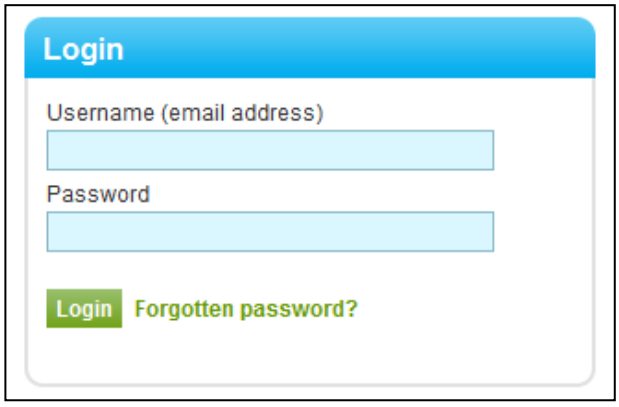
3 Accessing *UK PharmaScan*

3.1 Login

Once you have registered for *UK PharmaScan* and downloaded your lease, you can access the site by logging in with your username and password.

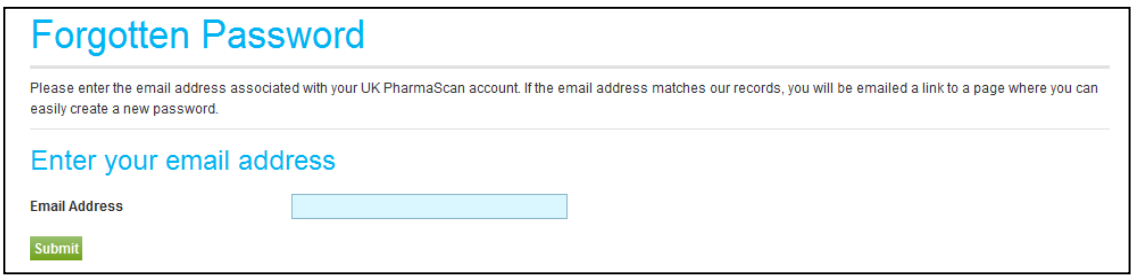
You must log in on the same PC you used to download your lease. If you have Flash installed on your PC, you will be able to login on any browser. If you do not have Flash installed, you can only log in using the same browser as the one you were using when you activated your account.

- ✓ Go to the *UK PharmaScan* website at: www.ukpharmascan.org.uk
- ✓ Enter your details in **Username (email address)** and **Password** fields
- ✓ Click the **Login** button



You will be taken to your homepage (section 4).

- ✓ If you have forgotten your password, click **Forgotten password?**
- ✓ Enter your email address in the box on the next page
- ✓ Click the **Submit** button



You will be sent an email containing a link to a page where you can reset your password.

3.2 Problems

If you do not log in to your account for a while, if you delete your Flash cookies (which store your lease information), or if your Flash software is updated, your lease will be deleted and you will not be able to log in.

If you try to log in you will be presented with a message telling you that no machine lease has been issued. Please contact the *UK PharmaScan* enquiries team (contactus@ukpharmascan.org.uk), and they will send you an email to reissue your lease.

3.3 Logout

✓ Click **Logout** in the top right corner of the page

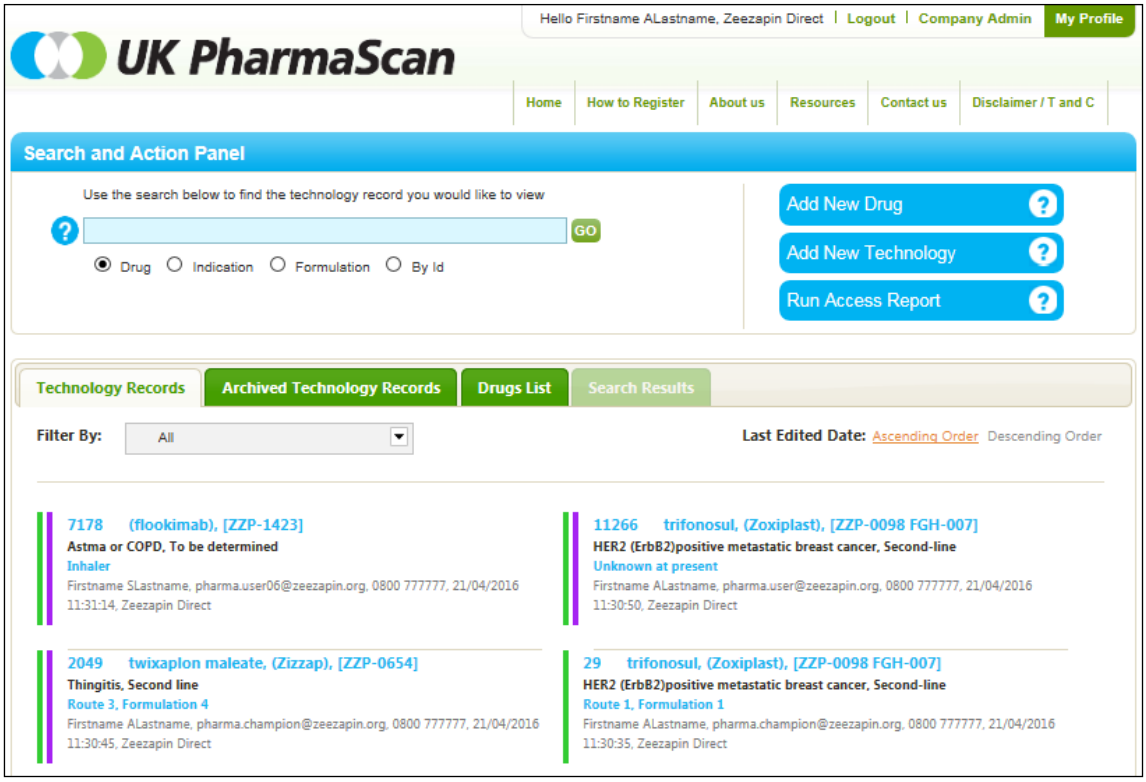
After logging out you will be returned to the standard homepage.

4 Navigation

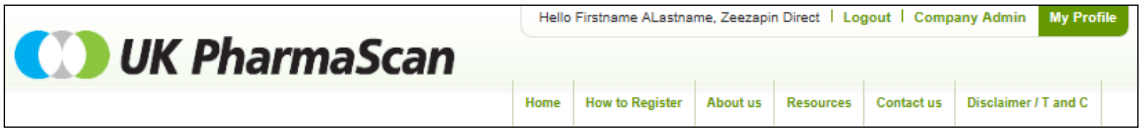
After logging in to *UK PharmaScan*, you will be taken to your homepage via a redirect page.

If you are not redirected automatically, click on **click here to go to your homepage**.

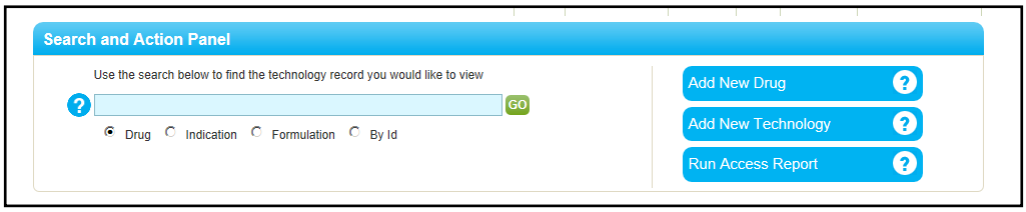
In the top right corner of your homepage, you will see your name and the name of your company, links to logout (section 3.3), the company admin pages (champion user only; section 12) and your profile information.



Below that, you will find the menu bar, with links back to the home page and a number of information pages.



The main body of your homepage is made up of two panels.
In the top panel you can search for records (section 8), add new drugs (section 5) and technologies (section 6) and run a horizon scanning access report (section 11.2).

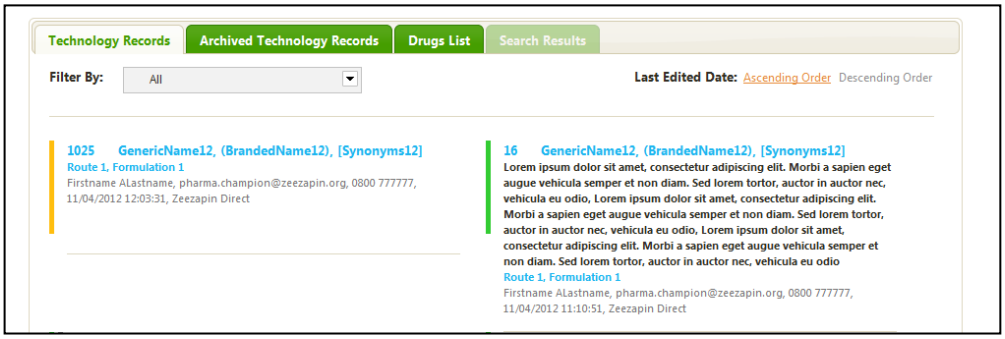


The lower panel contains four tabs:

- Technology Records
- Archived Technology Records
- Drugs List
- Search Results

The default view is the **Technology Records** tab.

✓ To view a different tab, click on the **tab name**



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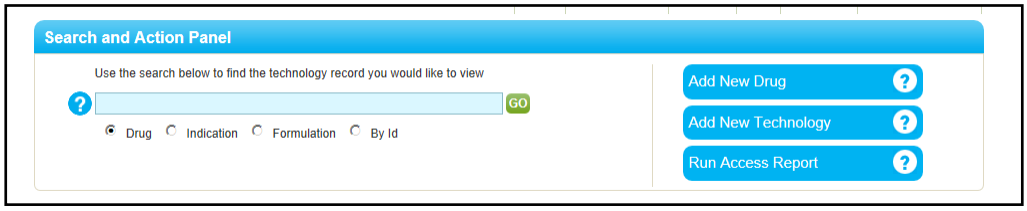
5 Drug records

5.1 Adding drug records

A drug record must be entered before a technology record can be entered.

✓ Click the **Add new Drug** button in the top panel on your homepage



✓ Enter the drug name, ensuring that you have completed *at least one* field

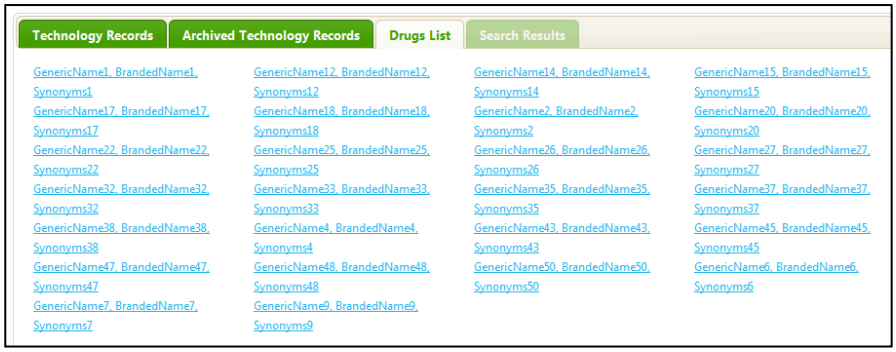
✓ Click the **Save** button to add the drug to *UK PharmaScan*

If a new *UK PharmaScan* record is created for medicines given in combination, you should ensure that all non-proprietary drug names are entered into the **Generic name** field. This will help horizon scanning organisations to differentiate medicines given as monotherapy from those given in combination.

Once you have saved the drug record, you can add an associated technology record (section 6).

5.2 Viewing and editing drug records

All drugs entered by your company are listed in the **Drugs List** panel in alphabetical order.



- ✓ To view a drug, click the **name**
- ✓ To edit a drug record, click the **name** and then click the **Edit Drug** button on the right hand side of the screen
- ✓ Edit the record
- ✓ Click the **Save** button

5.3 Deleting drug records

It is not possible to delete drug records from *UK PharmaScan*. If you have entered data incorrectly on a record which has **not yet been published**, and have other drugs to enter, you can reuse the record by editing it (section 5.2).

If you have published the drug record you should flag up to other users in your organisation that they should not re-use the record by entering 'ENTERED IN ERROR' in the **Generic name** field.

6 New technology records

6.1 Overview

When entering your data, please refer to the latest **quality assurance (QA) criteria**. If you do not have a copy of the criteria, a copy is available on the *UK PharmaScan* website Resources page or you can request it from the *UK PharmaScan* enquiries team (contactus@ukpharmascan.org.uk).

Examples of 'ideal' records for a product in Phase III and for a product for which the regulatory dossier has been submitted are available in appendices 1 and 2.

Guidance on adding clinical trials is available in section 7. Appendix 3 provides a list of all the fields and help text featured in *UK PharmaScan*. Lists of field response options are provided in appendix 4 (set dropdown options) and appendix 5 (set values). A list of disease states is available separately on request from the *UK PharmaScan* enquiries team (contactus@ukpharmascan.org.uk).

For certain fields, additional **guidance** and **examples** of the type of data expected are provided in tables in this section.

6.2 Creating technology records

Companies are asked to create and update technology records in a timely manner, and avoid retrospective completion of estimated date fields. Information on regulatory dates should be updated immediately the information becomes available and all other fields should be updated at least every 3 months. An automated email will be sent every 3 months after a record was last updated or marked as no change advising the record is due for review.

Existing records should not be re-used as each record has a unique ID number used by horizon scanning organisations. A new record must be created for each new product or indication. Existing records which are no longer required can be archived, see section 9.5 for more details on archiving records.

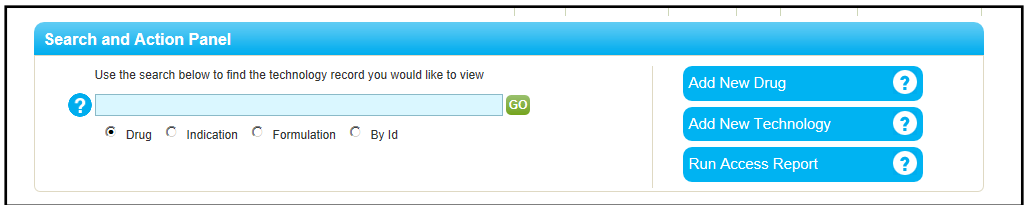
A retrospective technology record should not be created after a drug has already been launched as *UK PharmaScan* is not intended to be a source of information on products on the market in the UK.

If the product has multiple indications, or an existing drug has a new indication, create a new technology record for each indication. Do not use a single technology record for more than one indication. See section 9.2 for details on how to copy records.

6.3 Adding technology records

✓ Log in

✓ Click the **Add new Technology** button in the top panel on your homepage

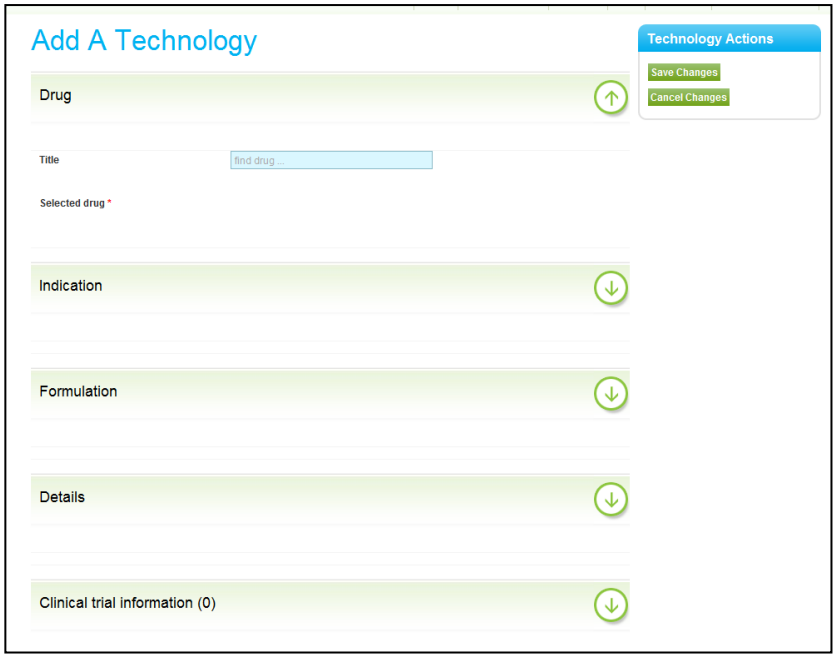


The **Add A Technology** form will be displayed. This is made up of 7 panels:

- Drug
- Indication
- Formulation
- Details
- Clinical trial information
- Regulatory information
- Cost and budgetary

Note that the technology record must be saved before clinical trial details can be added. Guidance on adding clinical trials is available in section 7.

✓ To open a panel, click the arrow on the right hand side of the panel name



- ✓ Enter as much data as possible

6.4 Requesting new values

It is recognised that for some of the fields the existing list of values may not be comprehensive. In these fields a **Request new value** option is available.

- ✓ Click the **Request new value** button
- ✓ Enter your suggestion
- ✓ Click **Submit your request**

These requests are reviewed quarterly by the *UK PharmaScan* User Group, which includes representatives from industry and the 7 national horizon scanning organisations.

New values approved by the User Group are automatically added to the relevant drop down list exactly as they are suggested. You should therefore keep your suggestion short, clear and free of typos.

Depending on when you submit your request, it can take up to 3 months for you to find out if it has been accepted. If your request is rejected, you will be told which existing option you should use instead.

6.5 Drug panel

The drug panel should already be open when you first open the technology form.

- ✓ Enter an existing drug name into the search box in the **Title** field. Start typing the drug name and a list of possible drug name records will be displayed.

- ✓ Select the correct drug

This will link the technology record to the drug record. If the search does not retrieve the drug you need, it has not been entered onto the database, and you will need to add a new drug record (section 5).

6.6 Indication panel

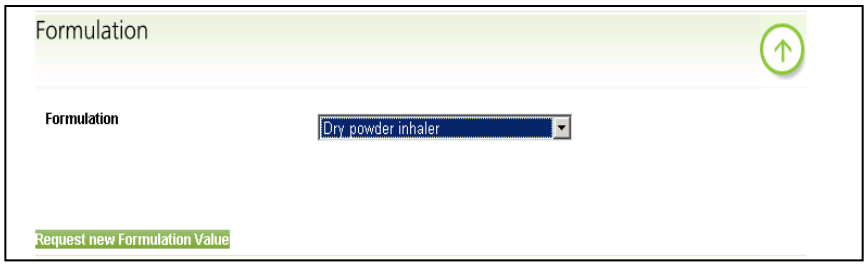
The **Abbreviated** and **Proposed place in therapy** fields are mandatory. It is not possible to save a record if they are left blank.

Field	Guidance
Final	Complete this field only when marketing approval has been obtained.
Abbreviated	Include the disease name and details of the involved organs, tissues, receptor and/or genes if appropriate. This field

	rather than the proposed indication field is displayed in search results.
Proposed place in therapy	Line of therapy, or other circumstances in which the drug is expected to be used
Is paediatric	Specify if the indication is for children or not. Create a separate technology record if there is also an adult indication.

Field	Example
Proposed	Drug A, in combination with Drug B, for the treatment of patients with advanced or metastatic breast cancer whose tumours are ER+ve. Patients should have progressive disease following prior therapy that must include anthracyclines and taxanes in the metastatic setting.
Final	Drug A, in combination with Drug B, for the treatment of patients with locally advanced or metastatic breast cancer whose tumours are ER+ve. Patients should have progressive disease following prior therapy that must include anthracyclines and taxanes in the metastatic setting.
Abbreviated	Breast cancer: advanced or metastatic ER+ve. (Further examples for 'Abbreviated' are available via the online help information.)
Proposed place in therapy	Second-line, following prior therapy with anthracyclines and taxanes. (Further examples for 'Proposed place in therapy' are available via the online help information.)

6.7 Formulation panel



If you cannot find an appropriate option from the drop-down list, you can use the drop-down value 'Other' and provide details in the **If other, please specify** in the free text box, or request a new formulation value by clicking the **Request new Formulation Value** button.

6.8 Details panel

The **Technology status** field is mandatory. It is not possible to save a record if it is left blank.

Field	Guidance
Mode of action	Include the pharmacological class.
Anticipated BNF class	Provide the fully qualified BNF Descriptor. Only a single BNF classification value may be entered - if other information is entered you will see a message stating, 'This is not a valid BNF class'.
Disease state	Search and select from the disease ontology. List available on request. Select the most specific term that covers the indications included in the licence application. Only use a more general term if no more specific term is available.
Is the drug considered a personalised medicine?	Indicate if the drug's use takes account of a person's genes, health, and environment.
Who is the originator company?	Where the originator company is different from your company, you can select from a list of companies registered with *UK PharmaScan*. If the originator company does not appear in the list, select 'Other' and type the name in the 'Originator company name' box
Co-marketed company	You can select from a list of companies registered with *UK PharmaScan*. If the co-marketing company does not appear in the list, select 'Other' and type the name in the 'Co-marketed company name' box

6.9 Clinical trial information

Guidance on adding clinical trials is available in section 7. If want to add clinical trial information, you must save the record first. If you start working on a clinical trial without saving the main record, you will lose any changes made.

6.10 Regulatory Information panel

Field	Guidance
Current EU stage of development	**Phase II** The product is the subject of a Phase II clinical trial but no Phase III trial has yet been started. **Phase III** The product is the subject of a Phase III clinical trial (possibly in parallel with a continuing Phase II trial) but no regulatory application has been made in the EU or a member state. **Pre-registration** A regulatory dossier has been filed with the European Medicines Agency, the MHRA or the national regulatory body of another member state, but a CHMP Opinion has not been issued and no marketing authorisation has been

	granted. Must be selected if a regulatory dossier has been submitted, even if Phase II or Phase III trials are ongoing. **CHMP Opinion** For products following the EU Centralised route, the Committee for Human Medicinal Products of the EMA has issued an Opinion on the product (positive or negative), but the EMA has not yet granted a marketing authorisation. **Licensed in member state** The product has received a marketing authorisation from the MHRA or the regulatory body of another member state, but has not yet been granted a marketing authorisation by the EMA under the Mutual Recognition procedure and has not yet been launched on the market in the UK. **Approved in EU** The product has been granted a marketing authorisation by the EMA, either under the Centralised or the Mutual Recognition procedure, but has not yet been launched on the market in the UK. **Available in UK** The product has received a marketing authorisation valid in the UK from either the EMA or the MHRA, has been launched on the market in the UK and may be prescribed within the product licence for the relevant indication and patient population.
Classified by EMA as an Advanced Therapy Medicinal Product (ATMP)?	Advanced Therapy Medicinal Product (ATMPs) are medicines that are based on genes, tissues or cells. All advanced therapy medicines are authorised centrally via the European Medicines Agency (EMA). They benefit from a single evaluation and authorisation procedure**.** If the EMA has agreed that the medicine is an ATMP, add the ATMP classification and the date that the decision was made.
MHRA Promising Innovative Medicine (PIM) designation granted?	The Early Access to Medicine Scheme (EAMS) is a voluntary MHRA regulated process that allows patients in the UK access to drugs intended for life-threatening or seriously debilitating conditions that do not yet have a marketing authorisation when there is a clear unmet need. It is a two-stage evaluation process. Step I involves receiving a Promising Innovative Medicines (PIM) designation. This will give an indication that a drug may be eligible for EAMS based on early clinical data. If a drug is not going through the EAMS process, or receives a negative PIM designation, then this field should be marked as 'No'.
Estimated Early Access to Medicines Scheme (EAMS) submission date	For products that have been awarded a PIM designation, an EAMS submission can be completed for MHRA consideration. Where possible please indicate the anticipated EAMS submission date using this field.

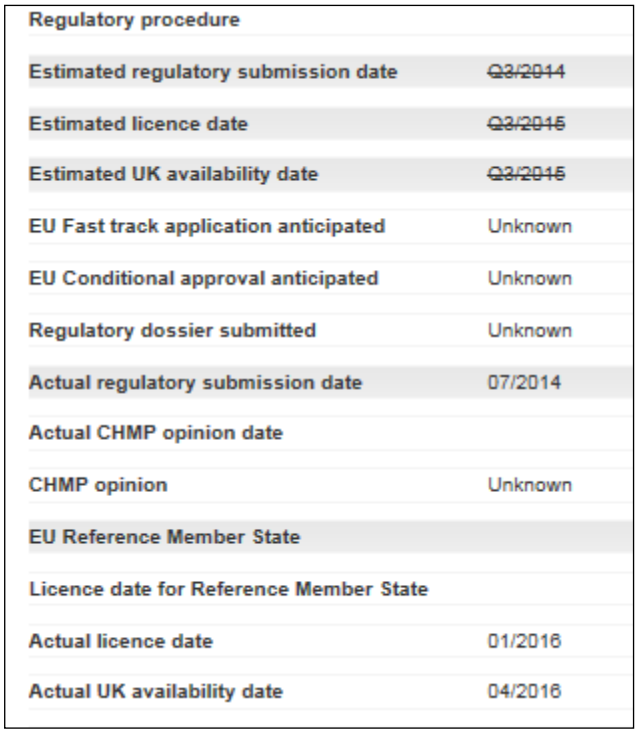
Actual EAMS submission date	
Estimated EAMS scientific opinion date	MHRA issues a scientific opinion on the benefits/risk balance of the drug. The opinion (which lasts for 1 year and can be renewed) supports the prescriber and patient to make a decision to use the drug before its licence is approved and does not replace normal licensing procedures.
Actual EAMS scientific opinion date	
EAMS scientific opinion decision	Include both the decision outcome and final wording regarding anticipated licence and population.
Regulatory procedure	**EU Centralised** This procedure results in a single marketing authorisation (called a 'community marketing authorisation') that is valid across the European Union, as well as in Iceland, Liechtenstein and Norway. The centralised procedure is compulsory for human medicines that are: • derived from biotechnology processes, such as genetic engineering • intended for the treatment of HIV/Aids, cancer, diabetes, neurodegenerative disorders or autoimmune diseases and other immune dysfunctions • officially designated 'orphan medicines' (medicines used for rare diseases). **EU mutual recognition** In the mutual-recognition procedure, a medicine is first authorised in one EU Member State (known as the reference member state). Following this, further marketing authorisations can be sought from other EU countries in a procedure whereby the countries concerned agree to recognise the validity of the original, national marketing authorisation. **MHRA** National authorisation procedure in the UK.
Estimated UK availability date	Indicate when the product is estimated to be available in the UK for supply against a prescription. Where there is no launch date available at the time a licence is granted, an estimated date should still be entered in this field.
Actual regulatory submission date	If this date is confidential check the 'Regulatory dossier submitted' tick box within 1 month of submission.
Actual CHMP opinion date	Indicate the actual CHMP opinion date. Prompt completion is essential for horizon scanning organisations managing the entry of new products into the NHS as it provides a specific timeframe for the issue of a marketing authorisation. Many

	horizon scanning organisations will use the CHMP opinion date as a prompt for active planning. Also complete details of the actual opinion (positive, negative etc.) and amend the estimated licensing date if necessary.
Actual UK availability date	Indicates date on which the product is made available in the UK for supply against a prescription. The technology entry will be archived 90 days from this date.
Information on EMA/MHRA decisions	Please provide a link to the SPC (and EPAR where available).
Suspension / EU discontinuation / archival	Use these fields if development of the product has been suspended or discontinued. If the MAA has been refused or if it is necessary to archive the technology record for some other reason (see section 9.5).
Development in the US / Response letter issued	Use these fields to provide details on whether the product has already been developed in the USA. Response letter issued, refers to the complete response letter issued by the US Food and Drug Administration.

When entering the estimated and actual dates for regulatory submission, licence and UK availability, it is important that the dates are entered in logical and chronological order and are kept up-to-date. **Estimated dates** should be in the future when the specified event (for example, regulatory submission date) is expected to occur. The **Estimated regulatory submission date** must be before the **Estimated licence date**, which must be the same or before the **Estimated UK availability date**. Where estimated dates have passed and actual dates have not been entered the estimated dates should be updated.

Actual dates (including **Actual CHMP opinion date**) should only be completed when the event has occurred. Again the dates are to be in a logical order, i.e. the **Actual regulatory submission date** must be before the **Actual licence date**, which must be the same or before the **Actual UK availability date**.

Once actual dates are completed, the equivalent estimated field will appear as strikethrough text on the web page and in pdf reports, for example, once the **Actual regulatory submission date** is completed, the **Estimated regulatory submission date** will appear as strikethrough text. It is not necessary to delete estimated dates when actual dates are completed.



Regulatory procedure	
Estimated regulatory submission date	~~Q3/2014~~
Estimated licence date	~~Q3/2015~~
Estimated UK availability date	~~Q3/2015~~
EU Fast track application anticipated	Unknown
EU Conditional approval anticipated	Unknown
Regulatory dossier submitted	Unknown
Actual regulatory submission date	07/2014
Actual CHMP opinion date	
CHMP opinion	Unknown
EU Reference Member State	
Licence date for Reference Member State	
Actual licence date	01/2016
Actual UK availability date	04/2016

6.11 Cost and Budgetary Information panel

It is acknowledged that providing cost estimates in advance of product launch is challenging and detailed costs may not be available until a few months before launch. This is, however, an essential aspect of horizon scanning processes as the cost projections support the NHS budget setting process (done in advance of the financial year in which the new product is likely to be launched) and ensure sufficient budget allocation.

Field	Guidance
Drug cost range	Provide the drug cost range calculated per patient per year or patient per episode if less than one year. If the ranges are too restrictive, use the 'Drug Cost Notes' to enter broader estimates or general information about proposed costing strategies. Costing estimates made by horizon scanning organisations should not be used to complete technology records.
Drug cost notes	The field can be used to provide details of estimated acquisition cost (or cost range) of the new product and the dosing regimen (or potential dose range) associated with the cost. Please specify what completed drug cost ranges refers to, i.e. whether it is per patient per year or per patient per episode.

	If accurate estimates are unavailable, a range, or 'ball-park' estimates are acceptable. Please indicate if the figures are accurate estimates or ball park figures. State clearly whether costs include or exclude VAT.
Is a Patient Access Scheme or alternative discount arrangement planned for this indication?	If a Patient Access Scheme or alternative discount arrangement i.e. from companies that are not part of the Pharmaceutical Price Regulation Scheme 2014 is planned, please tick all options that apply from England / Wales / England & Wales / Scotland / Northern Ireland.
Service impact	Indicate what the potential service impact (other than drug acquisition cost) of the new product may be to the NHS. For example, cost of testing or new equipment associated with its use; impact on staffing or service provision; administration of the drug. It is important to highlight whether the impact is expected to be significant.
Impact on patient and carers	Indicate the potential health impact of the new product, in terms of quality of life and survival. Include such aspects as patient preferences, adherence, and if possible, consider the wider societal health impact of the therapy.
UK patient population notes	Provide the incidence per 100,000, or for long term conditions prevalence per 100,000 or actual patient numbers. Where possible, data at the devolved level is preferential but if data is not known for all 4 regions then UK data should be specified. Reliable sources include guidance from the National Institute for Health and Care Excellence [NICE]) or Scottish data (e.g. epidemiology data from NHS National Services Scotland [NHS NSS] or Health Protection Scotland [HPS]) or Welsh data (e.g. www.infoandstats.wales.nhs.uk) and can be used to extrapolate estimates. Information on the epidemiology of the condition obtained through a systematic search of the published literature can be used to check the estimates derived from these epidemiological data or may be used instead, if these data are not available. Where the eligible population is estimated from an extrapolation of figures in published literature, population data should be taken from the latest mid-year population estimates. Details should be provided for any complex calculations and any assumptions used in calculating estimated patient population should be outlined. In all cases, please state **reference sources** used for epidemiological data. Epidemiology and costing estimates made by horizon scanning organisations should not be used to complete technology records.

Estimated eligible patient population	Provides details of the assumptions used to estimate the number of patients who would be eligible for treatment with the new product or licence. Enter details of any factors or issues that create uncertainty around the estimate of eligible population, for example, limitations of data used to estimate mean patient numbers for a rare condition or disease; or potential disparity in the distribution of patients across the UK for rare diseases with a genetic component. Please state any **reference sources** used and include details of any complex calculations.
Estimated uptake	Provides details of the proportion of the eligible population predicted to receive the new product in the first and fifth full calendar years after it is launched. Enter details of any other assumptions used to estimate uptake, for example, an assumption that the new product would only be given to a sub-group of eligible patients. Enter details of any factors or issues that create uncertainty around the estimate of uptake of the new product, for example, lack of data on the cost-effectiveness of the new product relative to relevant comparators, the potential for 'off-label' use in patients not covered by the indication in the proposed product licence, or proposed clinical guidance that may alter treatment pathways. Please state any **reference sources** used and include details of any complex calculations.
Estimated net incremental drug acquisition costs per annum at year 1 and 5	Provides the estimated net incremental drug acquisition costs. These are calculated by subtracting the estimated drug acquisition cost per annum of alternative treatment(s) from the estimated drug acquisition cost per annum of the new product. Enter details of the most common treatment(s) that the new product is likely to replace including dosing regimens. Enter details of the drug acquisition cost of the main treatment(s) that the new product would replace. Details should be provided for the source of costs for the alternative treatment(s) e.g. the most recent version of the BNF (www.bnf.org). Enter details of any factors or issues that create uncertainty around the estimate of incremental drug acquisition cost, for example, the pending loss of patent protection of an alternative treatment, or the pending launch of a similar new product. State clearly whether costs include or exclude VAT.
What will be the net budget impact at year 1 and 5?	Provides the net budget impact at year 1 and 5. This is calculated by multiplying the estimated uptake at years 1 and 5 by estimated net incremental drug acquisition cost per

	annum at years 1 and 5. State clearly whether costs include or exclude VAT.

Field	Example
UK patient population	*Example for drug A to treat adults with moderate to severe psoriasis unresponsive to standard therapies* Assumptions: • Estimated prevalent adult population in the UK with psoriasis of approximately 100,000 patients.
Estimated eligible patient population	*Example for drug A to treat adults with moderate to severe psoriasis unresponsive to standard therapies* Assumptions: • Approximately 2.5% of patients have moderate to severe disease. • Approximately 25% of these patients fail on standard therapies. Uncertainties: • The proportion of patients failing on standard therapies may alter over time with the introduction and greater use of biologic drugs as standard therapies. Estimated eligible patient population: • Estimated prevalent population of adults in the UK with psoriasis unresponsive to standard therapies of approximately 625 patients. References: • Reference 1 • Reference 2
Estimated uptake	*Example for drug A to treat adults with moderate to severe psoriasis unresponsive to standard therapies* Assumptions: Drug A would be given to approximately 5% and 15% of eligible patients in years 1 and 5, respectively. Uncertainties: Uptake may be influenced by cost-effectiveness relative to alternatives and this is unknown. Uptake may be influenced by the publication of new guidelines for psoriasis. Estimated uptake: Estimated uptake in years 1 and 5 by approximately 31 and 94 eligible patients, respectively.
Estimated net incremental drug acquisition costs per annum at year 1 and 5	*Example for drug A to treat adults with moderate to severe psoriasis unresponsive to standard therapies. Alternative treatments are drug B and drug C.* Assumptions:

	The estimated drug acquisition cost of drug A is approximately £10,000 to £12,000 per annum (30mg iv once a month), averaged at £11,000 per annum. Drug A would be used in place of drug B (25mg orally per day) and drug C (100mg sc per week). The alternative treatments (drug B and drug C) cost approximately £1,000 and £3,000 per annum, respectively. The average of these has been assumed as the cost of alternative treatments (£2,000 per annum). Uncertainties: Drug B is likely to be out of patent protection in the next few years and generic versions are expected to cost less, thus the incremental cost of drug A would increase. Estimated incremental drug acquisition cost: Estimated incremental drug acquisition cost of up to approximately £9,000 per annum.
What will be the net budget impact at year 1 and 5?	*Example for drug A to treat adults with moderate to severe psoriasis unresponsive to standard therapies* Year 1: (insert number of patients who will receive the new product) x £ (insert incremental drug acquisition cost [i.e. cost of new product – cost of alternatives]) = £ Year 5: (insert number of patients who will receive the new product) x £ (insert incremental drug acquisition cost [i.e. cost of new product – cost of alternatives]) = £ Year 1: 31 x £9,000 = £279,000 Year 5: 94 x £9,000 = £846,000

6.12 Saving the record

When you have entered your data, the record can be saved. Remember that it is not possible to save the record if the following fields have not been completed:

- Abbreviated indication
- Proposed place in therapy
- Technology status

✓ Click the **Save changes** button in the **Technology Actions** box

Once you have saved the changes, the page will refresh and the newly created record will be displayed. A unique identifying number will appear next to the **Technology Summary** heading and you will see options including 'Edit' and 'Submit to QA'.

Once you have saved your record, you can add details of clinical trials (section 7).

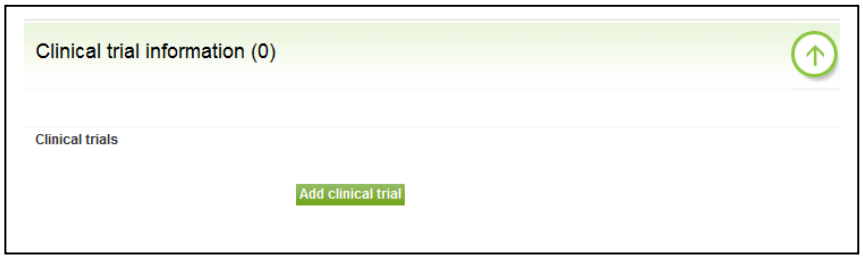
Please note, once all the known details for a record have been completed click the **Submit to QA** button. Records are not published on the website or visible to horizon scanning organisations until they have been submitted to and approved by QA.

7. Clinical trials

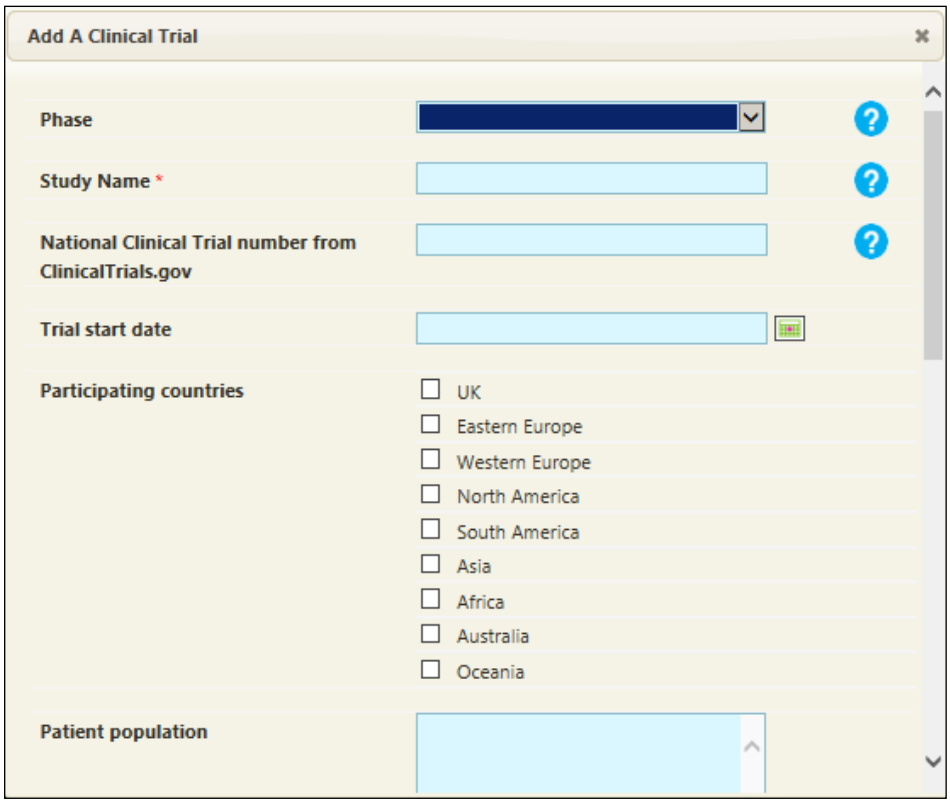
7.1. Adding clinical trials

To add clinical trial information, you must save the record first. If you start working on a clinical trial without saving the main record, you will lose any changes made.

- ✓ Expand the **Clinical trials information** panel
- ✓ Click the **Add clinical trial** button



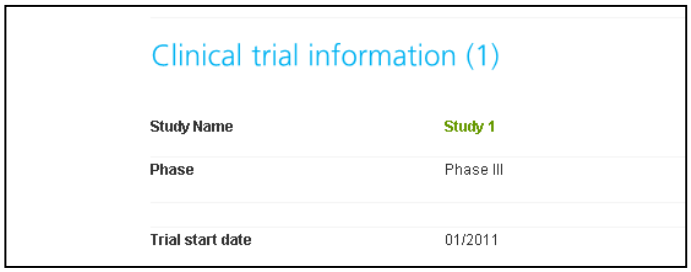
- ✓ Complete the **Add A Clinical Trial** form



The clinical trial information section should be completed with details of clinical trials used by the company to support its application for a marketing authorisation.

The clinical trials form asks users to enter a **Study Name** and the **National Clinical Trial number from ClinicalTrials.gov**. For trials listed on www.clinicaltrials.gov a hyperlink to the corresponding ClinicalTrials.gov record will be automatically generated when the National Clinical Trial number is entered (beginning with NCT) and the record saved.

Please note, only a valid NCT number will create a link to the corresponding ClinicalTrials.gov record. Any other number or phrase will still create a link but it will open up a browser window showing an error message. You can check if the links are working by saving your record and looking at it in view mode. The **Study Name** will be displayed **in green** and will link to www.clinicaltrials.gov.



If you do not publish data on www.clinicaltrials.gov you can enter web address links to other clinical trial registries or published trial documents in the **Publications** field.

Please note, horizon scanning organisations use the linked ClinicalTrials.gov record to access the full details of the trial, therefore the full trial information does not need to be duplicated in *UK PharmaScan*.

Field	Guidance
Study name	Study name – generally include the full name of the trial, plus any acronym or short name. This will become the hyperlink to the ClinicalTrials.gov record, which opens in a separate tab.
National Clinical Trial number from ClinicalTrials.gov	Enter with no spaces, e.g. NCT02101234. Do not enter more than one number or add additional text.
Trial start date	Field may be left blank if the information is available on the corresponding ClinicalTrials.gov NCT record.
Participating countries	Field may be left blank if the information is available on the corresponding ClinicalTrials.gov NCT record.
Patient population	Field may be left blank if the information is available on the corresponding ClinicalTrials.gov NCT record.

Study design	Field may be left blank if the information is available on the corresponding ClinicalTrials.gov NCT record.
Primary objectives	Field may be left blank if the information is available on the corresponding ClinicalTrials.gov NCT record.
Secondary outcomes	Field may be left blank if the information is available on the corresponding ClinicalTrials.gov NCT record.
Publications	List all relevant publications of trial results, including early publications such as company press releases and conference abstracts, although horizon scanning organisations will give more weight to papers in peer-reviewed journals. Include the URL and citation. If the trial is listed in another clinical trial registry, the number may also be included here, e.g. the International Standard Randomised Controlled Trial Number (ISRCTN) or the EU Clinical Trials Register (EudraCT) number.

- ✓ Click the **Save** button and **Submit to QA**

Please note, the information entered will not be published on the website or made available to the horizon scanning organisations until the **Submit to QA** button has been clicked and the record approved by QA.

7.2. Deleting clinical trials

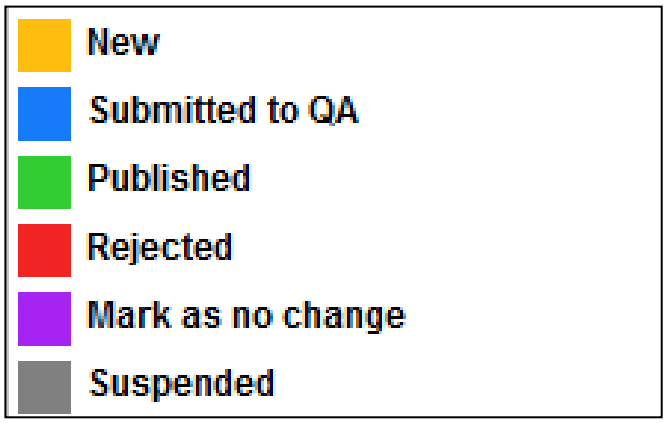
It is not possible to delete clinical trials from *UK PharmaScan*. If you have entered a trial incorrectly on a record **which has not yet been published**, and you have another trial to enter, you can reuse the trial fields by editing them.

If you have published the clinical trial information, you should clear all information from the record (it will remember the participating countries field) and enter 'ENTERED IN ERROR' in the **Study Name** field.

8. Viewing and searching for technology records

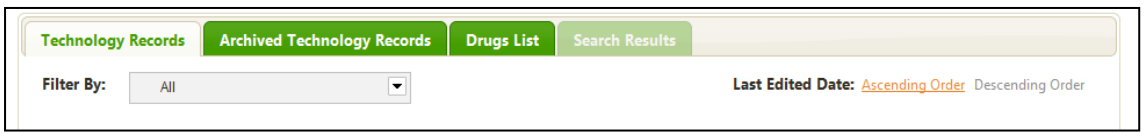
8.1. Viewing technology records

All technology records are colour coded according to their status.



Further information on the **Mark as no change** status is available in section 9.3.

Records can be filtered by status in the **Technology Records** tab. By default, records are displayed in **Descending Order** of the date they were last edited; that is, with the records edited most recently at the top. To change the display order so that the records edited most recently are at the bottom, click **Ascending Order**

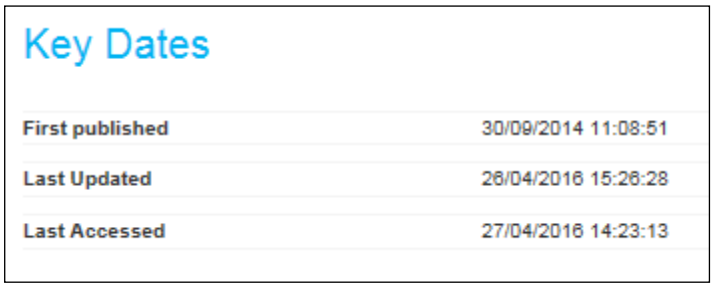


- ✓ Click the **Filter By** drop down list and select the status you want to view
- ✓ Click the **name** of the technology record to open it

The technology record displays all the entered details in the following order:

- Drug
- Indication
- Formulation
- Details
- Clinical trial information
- Regulatory information
- Cost and budgetary information

Key dates are displayed at the bottom of each record.



- **First published** date the technology record was first approved by QA and made visible to horizon scanning organisations
- **Last Updated** date the record was last edited and approved by QA. This includes when a record has been marked as no change and approved by QA
- **Last Accessed** date the record was last viewed by pharmaceutical or horizon scanning users

Audited changes are displayed in the audit history log at the bottom of edited technology records. This records the following information for each approved edit, including when a technology record is marked as no change:

- Field
- User name, Pharmaceutical company, Date and time
- Changed from:
- To

The audit history log is visible at the bottom of edited technology records both online and in pdf format for pharmaceutical users and online only for horizon scanning organisations.

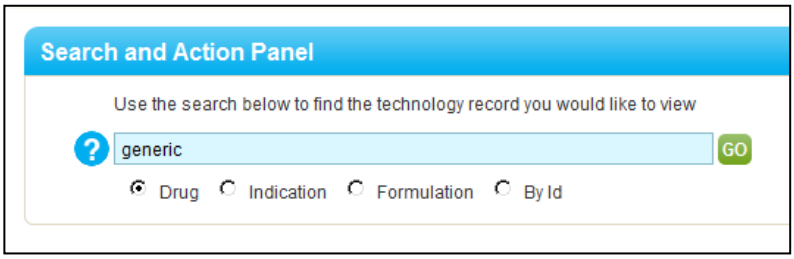
8.2. Searching for technology records

You can search for existing records by entering relevant search terms into the search box and selecting the field you want to search from:

- Drug name
- Indication
- Formulation
- Unique technology record ID number

✓ Select the field you want to search

- ✓ Enter your search term into the search box
- ✓ Click the **Go** button

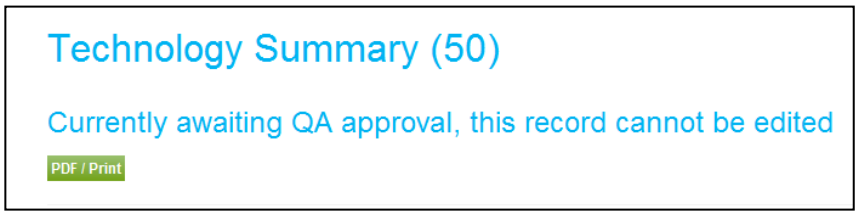


- ✓ Click the **name** of the technology record to open it

9. Editing, copying, deleting and archiving technology records

9.1. Editing technology records

Records which have the status **Submitted to QA** cannot be edited.



All other records can be edited.

- ✓ To edit a record, click the **name** of the technology record to open it

- ✓ Click the **Edit** button
- ✓ Edit the record
- ✓ Click the **Save changes** button in the **Technology Actions** box on the right hand side of the web page

If you are editing an existing record, and want to add or edit a clinical trial, you must save the record first. If you start working on a clinical trial without saving the main record, you will lose any changes made.

When you have made and saved all your changes click the **Submit to QA** button

so the record can be reviewed, approved and published. If you do not submit the record to QA the information entered will not be made available to the horizon scanning organisations.

Changes made to a technology record approved by QA are noted in the technology record audit history log.

An automated email will be sent every 3 months after a record was last updated or marked as no change advising the record is due for review. Please check all records and ensure these are up to date.

9.2. Copying records

The database enables you to copy data from an existing record into a new record for new indications and new formulations.

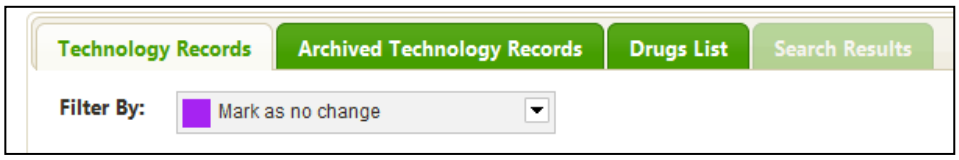
- ✓ Open the relevant technology record
- ✓ Click the **Copy (New Indication)** or **Copy (New Formulation)** button at the top of the record

9.3. Marking records as 'No Change'

When a technology record has not recently been updated, the database will change its status to **Mark as no change** and the record will be colour coded **purple**. You should review these records every three months to check whether or not they need to be updated.

Records which do not need to be updated should be marked as no change. Records marked as no change are automatically submitted to QA. Records which require updating should be edited (see section 9.1) and submitted to QA.

- ✓ Filter the records by **Mark as no change**



- ✓ Open each record
- ✓ Review the record
- ✓ If the record needs updating, edit and save it, and **Submit to QA**
- ✓ If the record does not require an update, click the **Mark as no change** button and the record will then be automatically submitted to QA.

The **Mark as no change** button is displayed on every record approved by QA and can be clicked at any time. Marking a record as no change is recorded in the technology record audit history log.

An automated email will be sent every 3 months after a record was last updated or marked as no change advising the record is due for review. Please check all records and ensure these are up to date.

9.4. Deleting technology records

It is not possible to delete technology records from *UK PharmaScan*. If you have entered data incorrectly on a record which has **not yet been published**, and have other technologies to enter, you can reuse the record by editing it (section 9.1).

If you cannot re-use an unpublished record, you should flag up to other users in your organisation and to horizon scanning organisations that the record is to be ignored by entering 'ENTERED IN ERROR' into the **Abbreviated indication** field (which appears on the homepage and in search results).

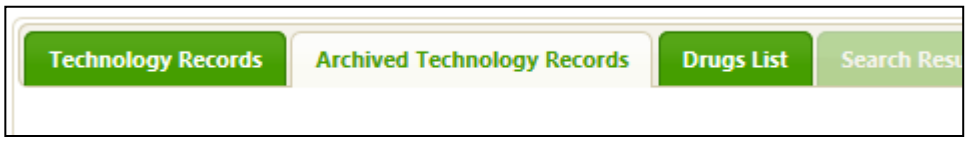
Unwanted records **which have been published** should be archived detailing the reason for archival (section 9.5); published records should NOT be re-used.

9.5. Suspending and archiving technology records

Records which have a date entered in **If suspended, date of suspension** will be marked as suspended and colour coded **grey**.

Records are automatically archived 90 days after the date completed in one of the following fields - **Actual UK availability date** or **If development in EU discontinued, date of discontinuation** or **If other reason for archival, date of**

decision to archive. The records will then appear in the Archived Technology Records tab on the homepage.



If development of a technology in the EU has been stopped for reasons other than launch, suspension or discontinuation, the date and reason can be completed in the Regulatory information section under **Other reason for archival**.

Other reasons for archival include:

- Drug is not being taken forward in UK due to commercial reasons
- Drug has been out-licensed to another company
- Rights to drug have been returned to the originator company

Please note, It is not possible to delete records from *UK PharmaScan* (see section 9.4).

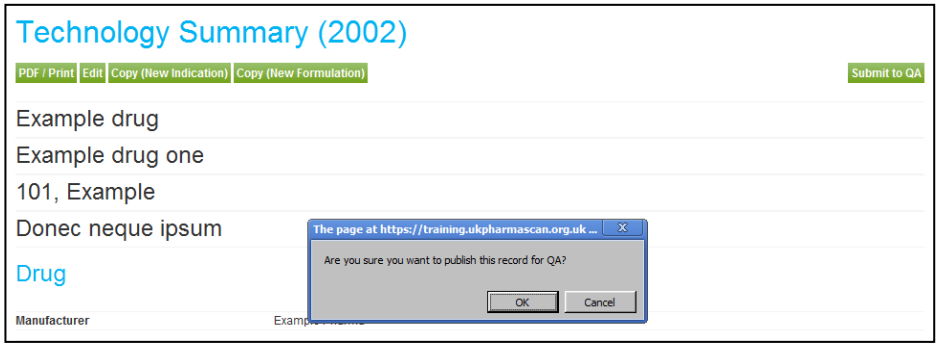
10. Quality Assurance

10.1. Submitting records to QA

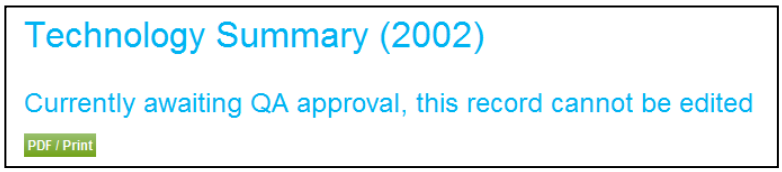
When entering your data, please refer to the latest **quality assurance (QA) criteria** to ensure the necessary information has been correctly entered. A copy is available on the *UK PharmaScan* website Resources page or can be requested from the *UK PharmaScan* enquiries team (contactus@ukpharmascan.org.uk).

When you have entered your drug and technology records and are ready to make them visible to horizon scanning organisations, you need to submit your records for QA.

- ✓ Click the **Submit to QA** button
- ✓ Click **OK** in the pop up box



The following message should now be displayed:



Once you click **Submit to QA** the technology record is colour coded **blue**. You cannot make any further changes to the record until it has been either approved or rejected by QA.

If the technology record is approved, you will receive an email informing you of this, the record will be published on the website and made visible to the horizon scanning organisations. The record status will be automatically changed to **Published** and colour coded **green**. Published records can be edited.

If the technology record is rejected, you will receive an email informing you of the reason for rejection. The record status will be automatically changed to **Rejected** and colour coded **red**.

Please note, a new technology record that has been rejected will not be visible to horizon scanning organisations until the record has been edited in line with the QA comments, resubmitted to QA and approved.

Please note, changes made to a published record that has been rejected will not be visible to horizon scanning organisations until the record has been edited in line with the QA comments, resubmitted to QA and approved. Until a record is approved by QA, the previously published version will remain visible to the horizon scanning organisations.

Changes made to technology records approved by QA are noted in the audit history log. This can be seen at the bottom of each technology record both online and in pdf format for pharmaceutical users and online only for horizon scanning organisations.

10.2. Records submitted in error

Records are only made available to horizon scanning organisations once they have been approved via QA. If a record is submitted for QA by mistake, please contact the *UK PharmaScan* enquiries team (contactus@ukpharmascan.org.uk) with the ID number of the record (the unique number that appears at the top of a technology record once you have saved it). The QA team will reject the record for you so you can amend and resubmit it.

11. Information on horizon scanning use of *UK PharmaScan*

11.1. Horizon scanning access

Seven approved horizon scanning organisations - NIHR Horizon Scanning Research & Intelligence Centre, NICE, NHS England (Specialised Services), UK Medicines Information, Scottish Medicines Consortium, All Wales Medicines Strategy Group, Health and Social Care Board (Northern Ireland) - have access to published records in *UK PharmaScan* to facilitate the provision of advice to the NHS regarding planning and providing support for the introduction into the NHS of new medicines and indications.

Horizon scanning organisations are required to sign a Data Accessor agreement and register a Champion User and Standard users totalling a maximum of 5 users as detailed in section 2.

The Data Accessor agreement details how information from *UK PharmaScan* must only be distributed in advice documents for use by the NHS in a format which either:

- presents only non-confidential information
- presents the data in a format which has been further analysed by the User such that such confidential information is not able to be discovered from reading the document
- clearly identifies any confidential information as such and places restrictions on readers as to the further non-disclosure of confidential information by the reader

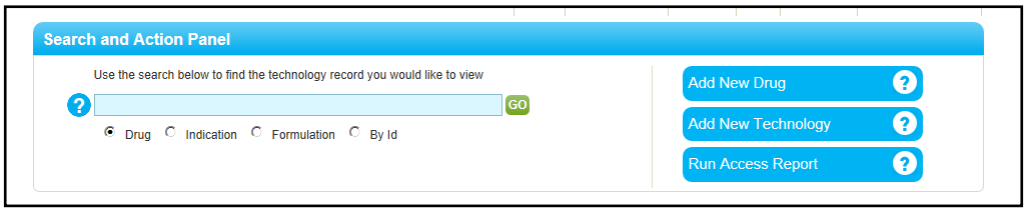
Horizon scanning users are able to run the following reports:

- **Custom Report** to view technology records published by pharmaceutical companies
- **Usage Report** to show companies who have added and have not added technologies to the database
- **Audit Report** to show all technology records that have been created or updated between a given start date and end date

11.2. Running a horizon scanning Access Report

Pharmaceutical companies are able to run an **Access Report** which details when your company's technology records have been accessed and by which horizon scanning organisation.

✓ Click the **Run Access Report** button in the top panel on your homepage



✓ Complete the details and click the **Run Report** button

On the search page you can choose to:

- search by a date range or search all dates
- search by a technology or search all technologies

The report will show the technology record ID, drug name, abbreviated name, horizon scanning organisation and date of access.

12. Champion User Administration

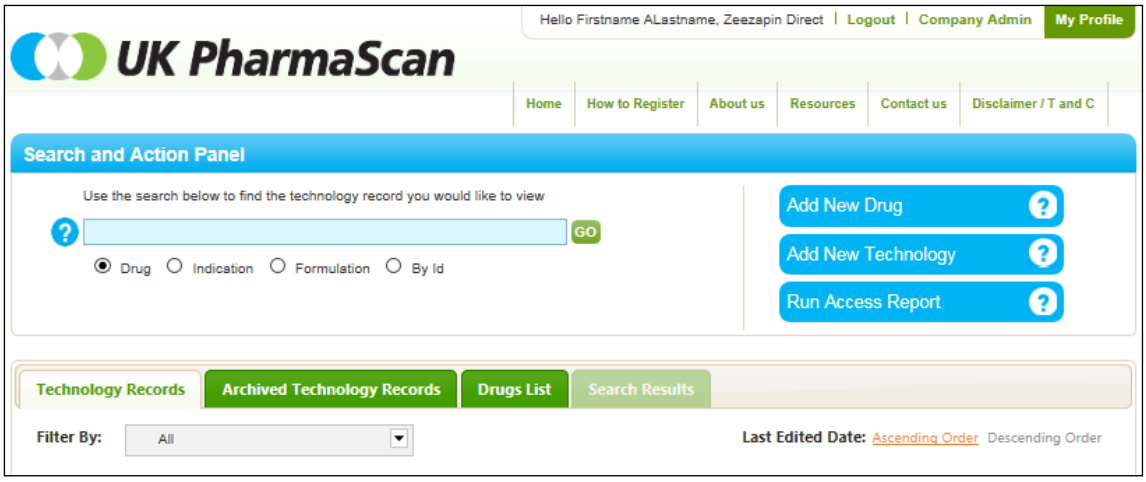
12.1. Company administration area

In the company administration area of the site, champion users can:

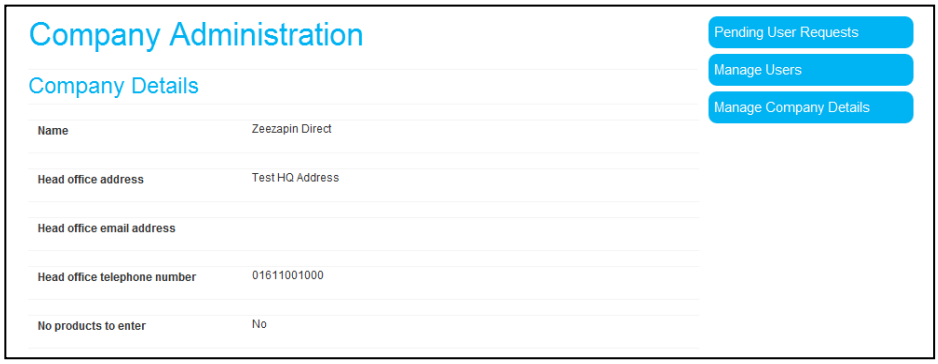
- Manage pending user requests (12.2)
- Manage users (12.3)
- Manage company details (12.4)

A maximum of 5 active users including the champion user are allowed at any one time. The QA team will periodically review user numbers and contact organisations with more than the maximum number.

✓ Click the **Company Admin** link in the top right corner of your homepage

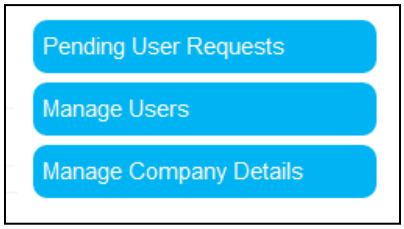


You will be presented with the company administration screen.

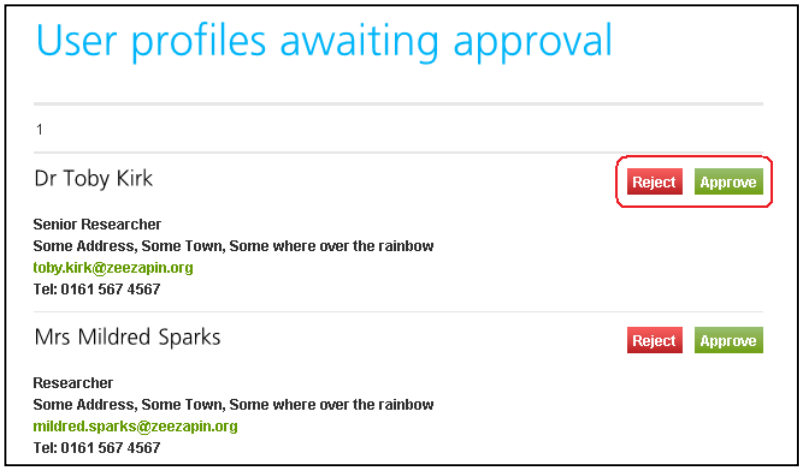


12.2. Managing user requests

✓ Click the **Pending User Requests** button



A list of pending user requests will be displayed.



✓ To approve a request, click the **Approve** button next to the user details

The system will automatically create a profile for the user in the system and send the user an activation email.

✓ To reject a request, click the **Reject** button next to the user details

The system will register the request and rejection in an audit log.

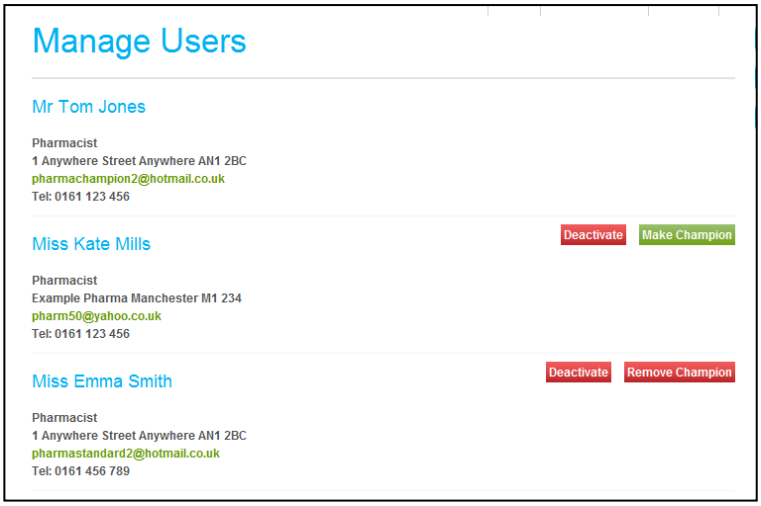
Once you have approved or rejected a request, the user record will be removed from the **User profiles awaiting approval** list.

12.3. Managing users

If you leave your organisation or go on leave, you need to allocate a new champion user using the **Manage Users** function. You can also use this function to activate or deactivate other users' accounts. If you deactivate a user's account they will be unable to log in.

✓ Click the **Manage Users** button

A list of all users for your company with *UK PharmaScan* accounts will be displayed on your **Manage Users** screen.



✓ To deactivate an account click the **Deactivate** button next to the user details

✓ To activate an account click the **Activate** button besides the user details

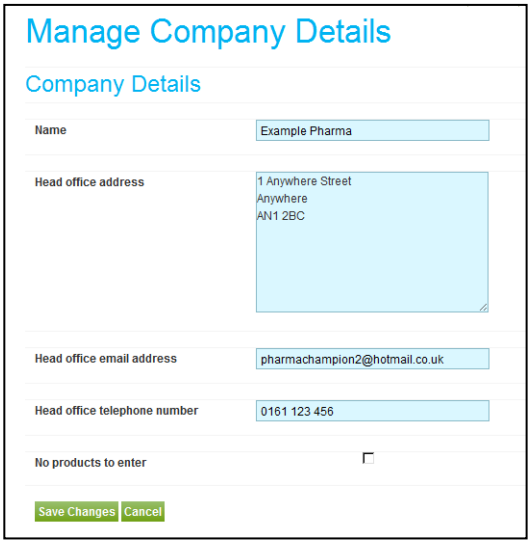
✓ To promote a standard user to a champion user, click the **Make Champion** button next to the user details

✓ To remove champion user permissions from a champion user, click the **Remove Champion** button next to the user details

12.4. Managing Company Details

You can edit all company details on the **Manage Company Details** form.

- ✓ Click the **Manage Company Details** button
- ✓ Edit details



- ✓ Click the **Save Changes** button

Your updated information will be displayed on the **Company Administration** page.

In addition to company details, if at registration stage you selected the **No products to enter** status, please ensure you keep this updated.

Appendix 1: Example of 'ideal' record when a product is first added to *UK PharmaScan* (in phase III or 3 years from launch in the UK)

Assura

Rifamilumab

Moderate to severe rheumatoid arthritis

Drug	
Manufacturer	Assurent Pharma Ltd
Branded name	Assura
Generic name	Rifamilumab
Synonyms	PC701, rifpuramab
Indication	
Proposed	In combination with methotrexate for the treatment of early arthritis.
Final	
Abbreviated	Early rheumatoid arthritis
Identified sub groups	Adults with RA and a symptom duration of 3-6 months
Proposed place in therapy	First-line treatment
Stage of disease	Early RA
Is paediatric	No
Formulation	
Formulation	Subcutaneous injection
Details	
Mode of action	Inhibitor of northodeconate dehydrogenase (NDDH), a key enzyme involved in joint destruction. First in class biological.
Technology status	New indication
Nature of SPC amendment	
Route	Parenteral
Presentation	Self-administered autoinjector containing 300mg rifamilumab in 1mL solution. Requires fridge storage.
Proposed dose	300mg
Proposed dosing regimen	Given by subcutaneous injection, initially 300mg at weeks 1 and 4, then every 6 months.
Anticipated BNF class	10.1.3
Disease state	Rheumatoid arthritis
Is the drug considered a personalised medicine?	No
Is there a companion diagnostic test?	No
Please provide details	
Current treatment options	Methotrexate; other DMARDs
Likely Comparators	As above

Has this medicine been formally selected for an AWMSG TDA?	Unknown
Comments	
Has this medicine been formally selected for a NICE HTA?	Unknown
Comments	
Will this medicine be appraised by the SMC?	Yes
Comments	
Who is the originating company?	Assurent Pharma Ltd
Is the drug being co-marketed?	No
Co-marketing company	
Clinical trial information	
Study Name	AS-104/9
National Clinical Trial number from ClinicalTrials.gov	NCT02101234
Phase	Phase III
Trial start date	
Participating countries	
Patient population	
Study design	
Primary objective	
Primary outcomes	
Secondary outcomes	
Anticipated date of study completion	Q4/2015
Anticipated date of study publication	
Publications	
Regulatory information	
Current EU stage of development	Phase III
Orphan drug status in EU	Unknown
Date Eu orphan drug status granted	
EU orphan number	
Classified by EMA as an Advanced Therapy Medicinal Product (ATMP)?	

MHRA Promising Innovative Medicine (PIM) designation granted?	No
Estimated Early Access to Medicines Scheme (EAMS) submission date	
Actual EAMS submission date	
Estimated EAMS scientific opinion date	
Actual EAMS scientific opinion date	
EAMS scientific opinion decision	
Regulatory procedure	EU Centralised
Estimated regulatory submission date	Q1/2016
Estimated licence date	Q3/2016
Estimated UK availability date	Q3/2016
EU Fast track application anticipated	No
EU Conditional approval anticipated	
Regulatory dossier submitted	
Actual regulatory submission date	
Actual CHMP opinion date	
CHMP opinion	
EU Reference Member State	
License date for Reference Member State	
Actual license date	
Actual UK availability date	
Information on EMEA/MHRA decisions	
MAA EU withdrawal date	
MAA EU withdrawal reason	
If suspended, date of suspension	
Reason for suspension	
Are there further plans for trials/refiling?	
If development in EU discontinued, date of discontinuation	

Reason for EU discontinuation	
If other reason for archival, date of decision to archive	
Other reason to archive	
Development in the US	Phase III
Response letter issued	
Date response letter issued	
FDA fast tracked	
FDA orphan drug status	No
Notes	
Cost and budgetary information	
Proposed average dose	300mg 6 monthly.
Place in therapy	Substitute for DMARDs
Estimated length of treatment	Ongoing
Drug cost range (per patient per year or patient per episode if less than one year)	£30,000 and £40,000
Drug cost notes	Excl. VAT, per patient per year
Is a Patient Access Scheme or alternative discount arrangement planned for this indication? If Yes, please tick all that apply.	
Comments	
Is the technology available on a compassionate basis pre-licence in the UK other than clinical trials	No
Service impact	Delayed radiographic progression may improve long-term prognosis. This may reduce burden on NHS.
Impact on patients and carers	It is anticipated that, if licensed, rifamilumab will decrease pain associated with early rheumatoid arthritis and potentially increase quality of life.
UK patient population range	Between 1,000 and 1,500 per 100,000
UK patient population notes	RA is the most common inflammatory polyarthropathy in the UK, affecting around 1% of the population (over 400,000 people in England and Wales). Ref: www.nice.org.uk/XX
Estimated eligible patient population	The disease is severe in around 15% of patients and its peak age of onset is 40-70 years Ref: www.nice.org.uk/XX
Is the drug an orphan drug?	No
Is the drug likely to have a significant service impact?	Unknown

Is the net budget impact for the UK greater than £5million at year 5?	Yes.
Estimated uptake	Details not available at this stage.
Estimated net incremental drug acquisition costs per annum at year 1 and 5	
What will be the net budget impact at year 1 and 5?	
Budget impact model available from the company on request	Unknown

Appendix 2: Example 'ideal' updated record for a product where a regulatory dossier has been submitted (1 year from launch)

Assura

Rifamilumab

Moderate to severe rheumatoid arthritis

Drug	
Manufacturer	Assurent Pharma Ltd
Branded name	Assura
Generic name	Rifamilumab
Synonyms	PC701, rifpuramab
Indication	
Proposed	In combination with methotrexate for the treatment of moderate to severe, active rheumatoid arthritis in adults for who the response to disease-modifying anti-rheumatic drug (DMARD) therapy, including methotrexate, has been inadequate
Final	
Abbreviated	Moderate to severe rheumatoid arthritis
Identified sub groups	Patients not adequately controlled on methotrexate/DMARDs
Proposed place in therapy	After the failure of two previous conventional disease modifying anti-rheumatic drugs including methotrexate
Stage of disease	Active moderate to severe RA
Is paediatric	No
Formulation	
Formulation	Subcutaneous injection
Details	
Mode of action	Inhibitor of northodeconate dehydrogenase (NDDH), a key enzyme involved in joint destruction. First in a new class of biological drugs.
Technology status	New chemical / biological entity
Nature of SPC amendment	
Route	Parenteral
Presentation	Self-administered autoinjector containing 300mg rifamilumab in 1mL solution. Requires fridge storage.
Proposed dose	300mg
Proposed dosing regimen	Given by subcutaneous injection, initially 300mg at weeks 1 and 4, then every 6 months.
Anticipated BNF class	10.1.3
Disease state	Rheumatoid arthritis

Is the drug considered a personalised medicine?	No
Is there a companion diagnostic test?	No
Please provide details	
Current treatment options	TNF-inhibitors such as adalimumab, certolizumab pegol, etanercept and golimumab.
Likely Comparators	As above
Has this medicine been formally selected for an AWMSG TDA?	No
Comments	AWMSG confirmed meets exclusion criteria for appraisal by AWMSG
Has this medicine been formally selected for a NICE HTA?	Yes
Comments	Wave 27, single technology appraisal.
Will this medicine be appraised by the SMC?	Yes
Comments	
Who is the originating company?	Assurent Pharma Ltd
Is the drug being co-marketed?	No
Co-marketing company	
Clinical trial information	
Study Name	PC-415-790
National Clinical Trial number from ClinicalTrials.gov	
Phase	Phase III
Trial start date	Q2/2010
Participating countries	Poland, Russia, South America
Patient population	225 adults with: documented RA diagnosis by the 1987 American College of Rheumatology (ACR) criteria for at least 6 months; disease duration ≥6 months (from symptom onset); failed 1 or more DMARD, including methotrexate; active RA despite current DMARD therapy; active disease defined as ≥4 tender and ≥4 swollen joints (out of 28 joints examined) and ESR ≥28 mm/hr or morning stiffness ≥45 minutes.
Study design	Randomised, double blind, placebo controlled for 12 months
Primary objective	Efficacy and safety of rifamilumab and methotrexate versus methotrexate monotherapy in subjects RA with inadequate response to a DMARD.

Primary outcomes	Number of subjects with an improvement of 20% in the American College of Rheumatology (ACR) Criteria at 3 months.
Secondary outcomes	Number of subjects with an improvement of 20% in the American College of Rheumatology (ACR) Criteria at week 52 Change from baseline in the Disability Index of the Health Assessment Questionnaire (HAQ) at week 52
Anticipated date of study completion	Q2/2012
Anticipated date of study publication	Q2/2014
Publications	Davis H, Randall C, McEntee J et al. Efficacy and safety of rifamilumab in moderate to severe rheumatoid arthritis: a randomised controlled study. Curr Res Opinion 2014; 38: 4-9
Study Name	PC-415-794
National Clinical Trial number from ClinicalTrials.gov	
Phase	Phase III
Trial start date	Q1/2007
Participating countries	UK, Western Europe, US, Australia
Patient population	Plan to enrol 754 adult patients with: documented RA diagnosis by the 1987 American College of Rheumatology (ACR) criteria for at least 6 months; disease duration ≥6 months (from symptom onset); failed 1 or more DMARD, including methotrexate; active RA despite current DMARD therapy; active disease defined as ≥4 tender and ≥4 swollen joints (out of 28 joints examined) and any one of the following: ESR ≥28 mm/hr; CRP ≥1.0 mg/dl; morning stiffness ≥45 minutes.
Study design	Randomised, double blind, placebo controlled for 12 months
Primary objective	Efficacy and safety of rifamilumab and methotrexate versus methotrexate monotherapy in subjects RA with inadequate response to a DMARD.
Primary outcomes	Number of subjects with American College of Rheumatology (ACR) Criteria improvement of 20%, 50% and 70% at 3 months.
Secondary outcomes	Number of subjects with an American College of Rheumatology (ACR) Criteria improvement of 20%, 50% and 70% at week 52 Change from baseline in Modified Total Sharp Score (TSS) at week 52 Change from baseline in the Disability Index of the Health Assessment Questionnaire (HAQ) at week 52
Anticipated date of study completion	Q2/2010

Anticipated date of study publication	Q3/2011
Publications	Press releases: July 2009 [www.assure.com/news/xxru12734.htl] Conference presentation: EULAR 2010: [OP0146] RIFAMILUMAB PLUS METHOTREXATE VS. METHOTREXATE MONOTHERAPY FOR MODERATE TO SEVERE RA: 52-WEEK RESULTS
Regulatory information	
Current EU stage of development	Pre-registration
Orphan Drug Status in EU	No
Date EU orphan drug status granted	
EU orphan status number	
Classified by EMA as an Advanced Therapy Medicinal Product (ATMP)?	No
MHRA Promising Innovative Medicine (PIM) designation granted?	No
Estimated Early Access to Medicines Scheme (EAMS) submission date	
Actual EAMS submission date	
Estimated EAMS scientific opinion date	
Actual EAMS scientific opinion date	
EAMS scientific opinion decision	
Regulatory procedure	EU Centralised
Estimated regulatory submission date	Q1/2014
Estimated license date	Q1/2015
Estimated UK availability date	Q1/2015
EU Fast track application anticipated	No
EU Conditional approval anticipated	
Regulatory dossier submitted	Yes
Actual regulatory submission date	01/2014

Actual CHMP opinion date	
CHMP opinion	
EU Reference Member State	
License date for Reference Member State	
Actual license date	01/2015
Actual UK availability date	
Information on EMEA/MHRA decisions	Originally submitted in May 2005 but withdrawn 16th June 2006: http://www.ema.europa.eu/docs/en_GB/document_library/Application_withdrawal_assessment_report//xxxxxxx.pdf . Plan to re-submit on the basis of a 2nd Phase III study.
MAA EU withdrawal date	Q2/2006
MAA EU withdrawal reason	Need for an additional clinical study to answer questions posed by EMA
If suspended, date of suspension	
Reason for suspension	
Are there further plans for trials/refiling?	Yes
If development in EU discontinued, date of discontinuation	
Reason for EU discontinuation	
If other reason for archival, date of decision to archive	
Other reason for archival	
Development in the US	Phase III
Response letter issued	Yes
Date response letter issued	Q3/2008
FDA fast tracked	Yes
FDA orphan drug status	No
General comments	
Cost and budgetary information	
Proposed average dose	300mg every 6 months
Place in therapy	Substitute
Estimated length of treatment	Ongoing
Drug cost range (per patient per year or patient per episode if less than one year)	£20,000 and £30,000
Drug cost notes	Inc. VAT Range above refers to ongoing costs (excluding year 1, which will be higher due to the initiation schedule for the drug)

Is a Patient Access Scheme or alternative discount arrangement planned for this indication? If Yes, please tick all that apply.	
Comments	
Is the technology available on a compassionate basis pre-licence in the UK other than clinical trials	No
Service impact	Substitute for anti-TNFs. Likely to be more expensive. However, following induction, administration is only required every 6 months, less frequently than that for the ant-TNFs. In addition, self-administration so no need for outpatient/GP visits for administration by a healthcare professional.
Impact on patients and carers	Reduced number of injections (every 6 months) and can be self-administered. Fewer visits to health facilities for administration purposes required vs. some of the alternative agents.
UK patient population range	Between 750 and 1,000 per 100,000
UK patient population notes	The estimated prevalence of rheumatoid arthritis in England is 0.86%, equivalent to around 346,000 people (NICE TA225 Rheumatoid arthritis (after the failure of previous anti-rheumatic drugs) - golimumab: costing statement, June 2011).
Estimated eligible patient population	The proportion of patients with RA who are eligible for treatment with biological drugs has been estimated as 10% of the prevalent population: approximately 34,600 people (NICE TA225 Rheumatoid arthritis (after the failure of previous anti-rheumatic drugs) - golimumab: costing statement, June 2011). Possibly 15% of the eligible patient population will receive rifamilumab at peak usage (Company estimate).
Is the drug an orphan drug?	No
Is the drug likely to have a significant service impact?	No
Is the net budget impact for the UK greater than £5million at year 5?	Yes
Estimated uptake	Possibly 15% of the eligible patient population will receive rifamilumab at peak usage (year 5) and uptake is likely to be approximately 5% at year 1. (Company estimate from internal data)
Estimated net incremental drug acquisition costs per annum at year 1 and 5	The estimated drug acquisition cost of rifamilumab is approximately £20,000 to £25,000 per annum (300mg every 6 months). Rifamilumab would be used in place of drug X (25mg subcutaneously every 2 weeks) and drug Y (100mg sc

	per week). The alternative treatments (drug X and drug Y) cost approximately £10,000 and £12,000 per annum, respectively. The average of these has been assumed as the cost of alternative treatments.
What will be the net budget impact at year 1 and 5?	
Budget impact model available from the company on request	Unknown

Appendix 3: *UK PharmaScan* - Fields

Field Name	Mandatory	Help Text	Comments
Drug			
Manufacturer	Automated		
Branded Name			
Generic Name	Yes	For combination products, please enter all non-proprietary drug names into the 'Generic name' field.	
Synonyms			
Indication			
Proposed		Example of proposed indication: Drug A, in combination with Drug B, for the treatment of patients with advanced or metastatic breast cancer whose tumours are ER+ve. Patients should have progressive disease following prior therapy that must include anthracyclines and taxanes in the metastatic setting.	
Final		Example of final indication: Drug A, in combination with Drug B, for the treatment of patients with locally advanced or metastatic breast cancer whose tumours are ER+ve. Patients should have progressive disease following prior therapy that must include anthracyclines and taxanes in the metastatic setting.	Final description of the indication. To be completed when marketing approval is obtained
Abbreviated	Yes	Examples of Abbreviated Indications and Proposed Place in Therapy – 11 examples listed	
Identified sub groups			
Proposed place in therapy	Yes	Examples of Abbreviated Indications and Proposed Place in Therapy – 11 examples listed	Description of the line of the therapy
Stage of disease			
Is paediatric		The drug has been developed for use in children only, or children too.	Yes/No/Unknown
Formulation			
Formulation	Yes		Drop down list: Options in Appendix 4
Details			
Mode of action			

Field Name	Mandatory	Help Text	Comments
Technology status	Yes		Drop down list: Options in Appendix 4
Nature of SPC amendment		Specify the section of the Summary of Product Characteristics (SPC) and state the change in full. If the change is to section 4.1, the technology status should be "New indication" rather than "Amendment to SPC with no change to licensed indication".	
Route	Yes		Drop down list: Options in Appendix 4
Presentation			
Proposed dose			
Proposed dosing regimen			
Anticipated BNF class		Only a single BNF classification value may be entered.	Only a single class may be added
Disease state			Search and select from the disease ontology. List available on request.
Is the drug considered a personalised medicine?		Choose the relevant response to indicate whether or not the drug takes account of a person's genes, health, and environment.	Yes/No/Unknown
Is there a companion diagnostic test?	Yes		Yes/No/Unknown
Please provide details			
Current treatment options			
Likely comparators			
Has this medicine been formally selected for an AWMSG TDA?			Yes/No/Unknown
Comments			
Has this medicine been formally selected for a NICE HTA?			Yes/No/Unknown
Comments			

Field Name	Mandatory	Help Text	Comments
Will this medicine be appraised by the SMC?			Yes/No/Unknown
Comments			
Who is the originator company?			Selected from a drop-down list of companies registered with *UK PharmaScan*
Originator company name			Only used if "Other" entered for previous field
Is the drug being co-marketed?			Yes/No/Unknown
Co-marketing company			Selected from a drop-down list of companies registered with *UK PharmaScan*
Co-marketing company name			Only used if "Other" entered for previous field
Clinical trial information			Details of multiple trials can be included.
Study name		Please provide details of any study name/acronym that the trial can also be identified by e.g. ADES, ELIOS	
National Clinical Trial number from ClinicalTrials.gov		Please enter the ClinicalTrials.gov identifier number which starts NCTXXXXXXXX - the system will use this to automatically create a link to the relevant trial on the ClinicalTrials.gov website. Please do not enter any other type of study code or information in this field as this will result in a broken link.	
Phase		Only details of the trials which support the licence are required. This will normally include only phase III trials but for those products likely to be fast tracked the details of phase II trials should be included.	Drop down list: Options in Appendix5
Trial start date			
Participating countries			Drop down list: Options in Appendix5
Patient population			
Study design			
Primary objective			
Primary outcomes			

Field Name	Mandatory	Help Text	Comments
Secondary outcomes			
Anticipated date of study completion		Date of study completion = date of last patients last study visit	
Anticipated date of study publication			
Publications		Within this section there is the opportunity to include details of relevant publications, press releases and abstracts for the interim and final results of this trial. For publications the full reference details should be provided (in the Vancouver style) along with a doi reference and/or a web address to the relevant journal.	With web links where available
Regulatory information			
Current EU stage of development	Yes	Please select current stage. After regulatory submission, please select the Pre-registration stage.	Drop down list: Options in Appendix 5
Orphan drug status in EU	Yes		Yes/No/Unknown
Date EU orphan drug status granted			Only visible if previous field completed
EU orphan status number			Only visible if previous field completed
Classified by EMA as an Advanced Therapy Medicinal Product (ATMP)?			Yes/No/Unknown
ATMP classification			Drop down list: Options in Appendix 5 Only visible if “Classified as an ATMP” field set to “Yes”
Date of recommendation on classification of ATMP			Only visible if “Classified as an ATMP” field set to “Yes”
MHRA Promising Innovative Medicine (PIM) designation granted?		The Early Access to Medicine Scheme (EAMS) is a voluntary MHRA regulated process that allows patients in the UK access to drugs intended for life-threatening or seriously debilitating conditions that do not yet have a marketing authorisation when there is a clear unmet need. It is a	Commercial in confidence field. Yes/No/Unknown

Field Name	Mandatory	Help Text	Comments
		two-stage evaluation process. Step I involves receiving a Promising Innovative Medicines (PIM) designation. This will give an indication that a drug may be eligible for EAMS based on early clinical data. Following designation the applicant is expected to complete a clinical development programme within a reasonable time period in order to progress to Step II, where the MHRA issue a scientific opinion on the benefits/risk balance of the drug. The opinion (which lasts for 1 year and can be renewed) supports the prescriber and patient to make a decision to use the drug before its licence is approved and does not replace normal licensing procedures.	
Estimated Early Access to Medicines Scheme (EAMS) submission date		As above	Commercial in confidence field.
Actual EAMS submission date		As above	Commercial in confidence field.
Estimated EAMS scientific opinion date		As above	Commercial in confidence field.
Actual EAMS scientific opinion date		As above	Commercial in confidence field.
EAMS scientific opinion decision		As above	Commercial in confidence field. Selected from drop-down list
Regulatory procedure		Please select the regulatory process being followed. **EU Centralised** This procedure results in a single marketing authorisation (called a 'Community marketing authorisation') that is valid across the European Union, as well as in Iceland, Liechtenstein and Norway. The centralised procedure is compulsory for human medicines that are: derived from biotechnology processes, such as genetic engineering; intended for the treatment of HIV/Aids, cancer, diabetes, neurodegenerative disorders or autoimmune diseases and other immune dysfunctions; officially designated 'orphan medicines' (medicines used for rare diseases) **EU mutual recognition** In the mutual-recognition procedure, a medicine is first authorised in one	Drop down list: Options in Appendix5

Field Name	Mandatory	Help Text	Comments
		EU Member State (known as the reference member state),. Following this, further marketing authorisations can be sought from other EU countries in a procedure whereby the countries concerned agree to recognise the validity of the original, national marketing authorisation. **MHRA** National authorisation procedure in the UK	
Estimated regulatory submission date		All estimated dates are commercial in confidence	Commercial in confidence field. Date must be less than 'Estimated licence date' and 'Estimated UK availability date'. Value struck-through once actual date is complete.
Estimated licence date	Yes	All estimated dates are commercial in confidence	Commercial in confidence field. Value struck-through once actual date is complete. Date must be same as or less than 'Estimated UK availability date'.
Estimated UK availability date	Yes	All estimated dates are commercial in confidence	Commercial in confidence field. Value struck-through once actual date is complete.
EU Fast Track application anticipated		Fast track is a reduced assessment time to 150 days (vs. 210 days).	Yes/No/Unknown
EU Conditional approval anticipated		Conditional approval is based on reduced clinical data, valid for 1 year (can be renewed) with obligation for ongoing studies	Yes/No/Unknown
Regulatory dossier submitted			Yes/No/Unknown.
Actual regulatory submission date		Date on which regulatory dossier was sent to regulatory agency. If this date is confidential please check tick box within 1 month of submission. This triggers the availability of this product monograph for the NHS user groups who subscribe to the database.	Commercial in confidence field. Date must less than 'Actual licence date' and 'Actual UK availability date'.
Actual CHMP opinion date			Only visible if 'Regulatory procedure' is 'EU centralised'

Field Name	Mandatory	Help Text	Comments
CHMP opinion			Only visible if 'Regulatory procedure' is 'EU centralised' Positive/Negative/Unknown
EU Reference Member State			Only for use if 'Regulatory Procedure' is 'EU Mutual Recognition'. Drop down list: Options in Appendix4
Licence date for Reference Member State			Only for use if 'Regulatory Procedure' is 'EU Mutual Recognition'.
Actual licence date			Date must be same as or less than 'Actual UK availability date'.
Actual UK availability date		This is the date upon which the product is made available in the UK for supply against a prescription - the technology entry will be archived 90 days from this date.	
Information on EMEA/MHRA decisions		Please provide a link to the SPC (and EPAR where available)	
MAA EU withdrawal date			
MAA EU withdrawal reason			Only visible if MAA EU withdrawal date entered.
If suspended, date of suspension			
Reason for suspension			Only visible if suspension date entered.
Are there further plans for trials/refiling?			Only visible if MAA EU withdrawal date entered. Yes/No/Unknown
If development in EU discontinued, date of discontinuation		The technology entry will be archived 90 days from this date.	
Reason for EU discontinuation			Only visible if discontinuation date entered.

Field Name	Mandatory	Help Text	Comments
If other reason for archival, date of decision to archive		If this record needs to be archived for a reason other than being discontinued or being made available in the UK, please enter the date and reasons. Other reasons for archival include: » Drug is not being taken forward in UK due to commercial reasons (but not available or discontinued) » Drug has been out-licensed to another company » Rights to drug have been returned to the originator company	
Other reason for archival			
Development in the US			Drop down list: Options in Appendix5 (US development stages)
Response letter issued			Yes/No/Unknown
Date response letter issued			
FDA fast tracked			Yes/No/Unknown
FDA orphan drug status			Yes/No/Unknown
General comments			
Cost and budgetary information			
Proposed average dose			
Place in therapy			Drop down list: Options in Appendix4
Estimated length of treatment			Commercial in confidence field
Drug cost range (per patient per year or patient per episode if less than one year)	Yes	It is acknowledged that providing a cost estimate up to one year in advance of product launch is challenging. This is, however, an essential aspect of horizon scanning processes as financial planners use the cost projections to support the budget setting process. This is done in advance of the financial year in which the new product is likely to be launched. Enter details of the estimated acquisition cost (or cost range) of the new product and the dosing regimen (or potential dose range) associated with this cost. If accurate estimates are unavailable, a range or 'ball-park'	Commercial in confidence field Drop down list: Options in Appendix5

Field Name	Mandatory	Help Text	Comments
		possible estimates are acceptable. Please indicate if the figures are accurate estimates or ball park figures. State clearly whether costs include or exclude VAT.	
Drug cost notes		If ranges in previous field are too narrow, please enter your own cost range here (minimum and maximum values).	Commercial in confidence field
Is a Patient Access Scheme or alternative discount arrangement planned for this indication? If Yes, please tick all that apply.		Patient access schemes are ways in which pharmaceutical companies can propose financial arrangements to enable patients to gain access to medicines. The Pharmaceutical Price Regulation Scheme 2014 makes provisions for companies to submit proposals for patient access schemes to the Department of Health. These schemes involve innovative pricing agreements designed to improve cost effectiveness and facilitate patient access to specific drugs or other technologies. Companies that are not part of the PPRS can submit proposals for similar alternative discount arrangements. Please add any additional detail to the 'Comments' free text field below.	Commercial in confidence field England/Wales/England & Wales/Scotland/Northern Ireland
Comments			Commercial in confidence field
Is the technology available on a compassionate basis pre-licence in the UK other than clinical trials?			Yes/No/Unknown
Service impact		Please indicate what the potential impact (other than drug acquisition cost) of the new product may be to the NHS. For example, cost of testing or new equipment associated with its use; impact on staffing or service provision. Note whether the impact is expected to be significant.	
Impact on patients and carers		Please provide information on potential health impact of the new product, in terms of quality of life and survival. Include such aspects as patient preferences, adherence and if possible consider the wider societal health impact of the therapy.	
UK patient population range	Yes	Select a range representative of the UK patient population range of the new product. Please provide accurate estimates, along with additional information in the free text 'UK patient population notes' field.	Drop down list: Options in Appendix 5

Field Name	Mandatory	Help Text	Comments
UK patient population notes		Where possible UK data should be used but if this is not available, English or Welsh data from reliable sources (e.g. guidance from the National Institute for Health and Care Excellence [NICE]) or Scottish data (e.g. epidemiology data from NHS National Services Scotland [NHS NSS] or Health Protection Scotland [HPS] or Welsh data (e.g. http://www.infoandstats.wales.nhs.uk/) should be used and extrapolated to produce estimates. Information on the epidemiology of the condition obtained through a systematic search of the published literature can be used to check the estimates derived from these epidemiological data or may be used instead, if these data are not available. Please state reference sources used for epidemiological data. Where the eligible population is estimated from an extrapolation of figures in published literature, population data should be taken from the latest mid-year population estimates. Details should be provided for any complex calculations and any assumptions used in calculating estimated patient population should be outlined.	State reference sources used for epidemiological data. Details should be provided for any complex calculations and any assumptions used in calculating estimated patient population should be outlined.
Estimated eligible patient population		Enter details of assumptions used to estimate the number of patients who would be eligible for treatment with the new product or licence. Enter details of any factors or issues that create uncertainty around the estimate of eligible population, for example, limitations of data used to estimate mean patient numbers for a rare condition or disease; or potential disparity in the distribution of patients across the UK for rare diseases with a genetic component. Please state any reference sources used and include details of any complex calculations. One example given.	State any reference sources used and include details of any complex calculations.
Is the drug an orphan drug?			Yes/No/Unknown
Is the drug likely to have a significant service impact?			Yes/No/Unknown

Field Name	Mandatory	Help Text	Comments
Please specify			Only visible if 'Yes' selected in previous field
Is the net budget impact for the UK greater than £5million at year 5?			Commercial in confidence field Yes/No/Unknown
Estimated uptake			Commercial in confidence field Only visible if 'Yes' selected in previous field State any reference sources used and include details of any complex calculations.
Estimated net incremental drug acquisition costs per annum at year 1 and 5			Commercial in confidence field Only visible if 'Yes' selected in net budget field
What will be the net budget impact at year 1 and 5?			Commercial in confidence field Only visible if 'Yes' selected in net budget field
Budget impact model available from the company on request			Commercial in confidence field Only visible if 'Yes' selected in net budget field Yes/No/Unknown.

Appendix 4: *UK PharmaScan* – Current dropdown options.

Last updated 23 July 2018

Formulation	Details		Regulatory Information	Cost and Budgets
Formulation	**Technology status**	**Route**	**EU reference member state**	**Place in therapy**
Buccal tablet	Biosimilar	Enteral	Austria	Add on therapy
Capsule	New chemical /	Inhaled	Belgium	No other treatment apart from
Chewable tablet	biological entity	Parenteral	Bulgaria	best support care
Cream	New dosing regimen	TBC	Croatia	Other
Cutaneous solution	New formulation	Topical	Cyprus	Substitute
Dispersible tablet	New indication		Czech Republic	
Dry powder inhaler	New presentation		Demark	
Ear drops			Estonia	
Enema			Finland	
Enteric coated tablet			France	
Eye drops			Germany	
Gel			Greece	
Granules			Hungary	
Implant			Ireland	
Inhalation powder			Italy	
Inhaler			Latvia	
Intra-articular injection			Lithuania	
Intradermal injection			Luxembourg	
Intralesional injection			Malta	
Intramuscular injection			Netherlands	
Intra-ocular injection			Poland	
Intrathecal injection			Portugal	
Intratumoural injection			Romania	
			Slovakia	

Intrauterine device (when carrying drugs e.g. progesterone) Intravenous infusion Intravenous injection Intravitreal injection Long-acting release (LAR) depot injection Metered dose inhaler Modified release capsule Modified release tablet Nasal spray Nose drops Ocular implant Ointment Oral formulation Oral solution Oral suspension Oromucosal solution Other Parenteral formulation Pessary Powder for oral solution Powder for solution for infusion Powder for solution for injection Sealant powder Solid oral dosage formulation Soluble capsule Solution for nebuliser Subcutaneous infusion			Slovenia Spain Sweden United Kingdom	

Subcutaneous injection Sublingual spray Sublingual tablet Suppository Tablet Topical implant Topical solution Transdermal patch Transdermal systems Unknown at present				

Appendix 5: *UK PharmaScan* – Set values

Clinical trial information		Regulatory information				
Phase	**Participating countries**	**Current EU stage of development**	**EAMS scientific opinion decision**	**Regulatory procedure**	**Development in the US**	**ATMP classification**
Phase II Phase III	UK Eastern Europe Western Europe North America South America Asia Africa Australia Oceania	Phase I Phase II Phase III Pre-registration CHMP opinion Licenced in member state Approved in EU Available in UK	Positive Negative	EU Centralised EU Decentralised EU Mutual recognition MHRA	Phase II Phase III Filed Recommended for approval Approved Launched Not recommended for approval	Tissue engineered medicinal product Gene therapy medicinal product Somatic cell therapy medicinal product

Cost and Budgets	
Drug cost range	**UK Patient population range**
<£1,000 £1,000 and £5,000 £5,000 and £10,000 £10,000 and £20,000 £20,000 and £30,000 £30,000 and £40,000 £40,000 and £50,000 £50,000 and £100,000 >£100,000 Not known	Less than 1 per 50,000 Between 1 per 50,000 and 25 per 100,000 Between 25 and 50 per 100,000 Between 50 and 150 per 100,000 Between 150 and 250 per 100,000 Between 250 and 500 per 100,000 Between 500 and 750 per 100,000 Between 750 and 1,000 per 100,000 Between 1,000 and 1,500 per 100,000 Between 1,500 and 2,000 per 100,000 Between 2,000 and 3,000 per 100,000 Over 3,000 per 100,000 Not known