UK PharmaScan

Example of '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.
BNF Chapter	10 – Musculoskeletal and joint diseases
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	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	Q1/2019
Anticipated date of study publication	Q4/2019
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-415-790
National Clinical Trial number from ClinicalTrials.gov	
Phase	Phase III
Trial start date	Q2/2018
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	Q4/2019
Anticipated date of study publication	Q2/2020
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
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 (quarter)	Q1/2021
Estimated regulatory submission date (month)	January
Estimated licence date (quarter)	Q3/2021
Estimated licence date (month)	August
Estimated UK availability date (quarter)	Q3/2021
Estimated UK availability date (month)	August
EU Fast track application anticipated	No
EU Conditional approval anticipated	
Regulatory dossier submitted	Yes
Estimated CHMP opinion date	Q1/2021
Actual CHMP opinion date	
CHMP opinion	

Actual regulatory submission date	
EU Reference Member State	
Licence date for Reference Member State	
Actual licence date	
Actual UK availability date	
Information on EMA/MHRA decisions	Originally submitted in May 2018 but withdrawn 16 June 2019: 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	Q2/2019
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 to archive	
Development in the US	Phase III
Response letter issued	Yes
Date response letter issued	Q3/2019
FDA fast tracked	Yes
FDA orphan drug status	No
General comments	
Cost and budgetary information	
Proposed average dose	300mg 6 monthly.
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?	
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 2019).
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 2019). 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