SMC Scottish Medicines Consortium
Advising on new medicines for Scotland
www.scottishmedicines.org.uk
NHS SCOTLAND
Delta House • 50 West Nile Street • Glasgow • G1 2NP • Tel 0141 225 6999 • Chairman: Dr Alan G MacDonald

conestat alfa 2,100 units powder (and solvent) for solution for injection (Ruconest®)

SMC No 745/11

Pharming Group NV

6 July 2018

The Scottish Medicines Consortium (SMC) has completed its assessment of the above product and advises NHS Boards and Area Drug and Therapeutic Committees (ADTCs) on its use in Scotland. The advice is summarised as follows:

ADVICE: following a full submission assessed under the ultra-orphan medicine process

conestat alfa (Ruconest®) is accepted for use within NHSScotland.

Indication under review: For treatment of acute angioedema attacks in adults and adolescents with hereditary angioedema (HAE) due to C1 esterase inhibitor deficiency.

Conestat alfa was associated with a significantly shorter time to relief from symptoms of HAE attack compared with placebo during controlled phase III studies.

This SMC advice takes account of the benefits of a Patient Access Scheme (PAS) that improves the cost-effectiveness of conestat alfa. This advice is contingent upon the continuing availability of the PAS in NHS Scotland or a list price that is equivalent or lower.

This advice takes account of views from a Patient and Clinician Engagement (PACE) meeting.

Chairman,
Scottish Medicines Consortium

Published 13 August 2018

Indication

For treatment of acute angioedema attacks in adults and adolescents with hereditary angioedema (HAE) due to C1 esterase inhibitor deficiency.[1, 2]

Dosing Information

The recommended dose for patients weighing up to 84kg is one slow intravenous injection of 50 units/kg body weight.
The recommended dose for patients weighing ≥84kg is one slow intravenous injection of 4,200 units (two vials).

In the majority of cases a single dose of conestat alfa is sufficient to treat an acute angioedema attack. In case of an insufficient clinical response, an additional dose (50 units/kg body weight up to 4,200 units) can be administered. Not more than two doses should be administered within 24 hours.

Conestat alfa should be initiated under the guidance and supervision of a physician experienced in the diagnosis and treatment of hereditary angioedema. Conestat alfa must be administered by a healthcare professional until the patient (or caregiver) is competent to administer after having been properly trained and in agreement with the healthcare professional.[1, 2]

Product availability date

Powder for solution for injection available October 2011; powder and solvent for solution for injection anticipated February 2018

Conestat alfa meets SMC ultra-orphan criteria

Background

Hereditary angioedema (HAE) is a rare autosomal dominant disorder that affects the gene responsible for the production of C1 esterase inhibitor which leads to functional levels in plasma below 50% of normal. HAE is characterised by recurrent, often unpredictable attacks of angioedema resulting in subcutaneous and submucosal swelling in any part of the skin and in the respiratory and gastrointestinal tracts. Attacks affecting the upper airways may be life-threatening. HAE cannot be cured but medication can be used to treat and prevent attacks.[3, 4]

Conestat alfa is a recombinant C1 esterase inhibitor which is produced from the milk of rabbits expressing the gene encoding for human C1 esterase inhibitor. It was initially marketed as a powder for solution for injection. In 2017, an additional presentation of conestat alfa received marketing authorisation. This 2,100 unit powder for solution for injection comes in a complete kit package including conestat alfa powder vial, solvent vial and administration devices (syringe, vial adaptors, infusion set and needle) which is intended to facilitate administration by the patient or the caregiver in the home care setting (home-treatment/self-administration). Both presentations require intravenous administration.[1, 2]

Conestat alfa for use in this indication has been considered by SMC using its decision-making framework for the assessment of ultra-orphan medicines.

Nature of condition

HAE is a rare condition and estimates of prevalence range from 2 to 3 in 10,000 to 1 in 50,000 of the population.[3, 4] The management of HAE includes treatment of acute attacks, short-term prophylaxis to prevent an attack following a trigger (e.g. surgical or dental procedures) and long-term prophylaxis to minimise the frequency and severity of recurrent attacks.[3, 4] Currently available treatments for acute attacks include plasma-derived C1 esterase inhibitors (Berinert® and Cinryze®) or icatibant (bradykinin receptor antagonist) which blocks the vasodilatating effects of bradykinin during an HAE attack.[3, 4] Conestat alfa is a recombinant C1 esterase inhibitor. Conestat alfa meets SMC ultra-orphan criteria

A patient and clinician engagement (PACE) meeting was held to consider the added value of conestat alfa in the context of treatments currently available in NHS Scotland. At the PACE meeting, attention was drawn to the unpredictable variability in the severity of HAE attacks which can be life threatening and considered a medical emergency if the airways are involved. The frequency of attacks can also vary significantly between and within individual patients. Some patients experience more than two attacks each week which may each last several days. These patients are confined to bed or home and are unable to manage normal daily living, caring for family members or work. Although some patients have infrequent attacks, the unpredictability in the timing and severity of attacks can be significantly disruptive to health, family and social life, work and education. These situations have a substantial negative impact on quality of life.

Impact of new technology

Summary of evidence on comparative efficacy

The key evidence comes from three controlled phase II/III studies which compared conestat alfa with placebo in patients with HAE. These included two similarly designed studies (C1-1205-01 and C1-1304-01) conducted in North America and Europe respectively and one world-wide study (C1-1310). Eligible patients were aged ≥12 years in study C1-1205-01, ≥16 years in study C1-1304 and ≥13 years (≥18 years outwith North American centres) in study C1-1310. They had a confirmed diagnosis of HAE with a baseline plasma level of functional C1 esterase inhibitor <50% of normal. Screened patients were randomised to study treatment provided they presented within five hours of onset of an attack affecting the peripheral, abdominal, facial and/or oropharyngeal/laryngeal area. At presentation and pre-dosing, the attack had an overall severity visual analogue scale (VAS) score ≥50mm with no improvement in symptoms (defined as a decrease ≥20mm in VAS score). The VAS assessment scored the overall severity of angioedema symptoms on a 100mm continuous scale where 0mm = no symptoms and 100mm = extremely disabling. In studies C1-1205-01 and C1-1304-01, patients were randomised to conestat alfa 100 units/kg (unlicensed dose, which will not be discussed further) or saline. Study C1-1205-01 also included a conestat alfa 50 units/kg group. In study C1-1310, patients were randomised to conestat alfa 50 units/kg for patients weighing <84kg or 4,200 units for patients weighing ≥84kg (licensed dose) or saline.

In all studies, the primary outcome was the time to beginning of symptom relief and the key secondary outcome was the time to minimal symptoms but these were defined differently across studies.

In studies C1-1205-01 and C1-1304-01, the primary outcome was defined as ≥20mm decrease from baseline in overall severity VAS scored on two consecutive assessments. In study C1-1205-01, this was significantly shorter in the conestat alfa 50 units/kg group (n=12) compared with placebo (n=13): median time to beginning of symptom relief of 122 minutes and 258 minutes respectively, p<0.001. The time to minimal symptoms was defined as the time at which all overall severity VAS scores fell below 20mm for all locations and was a median of 247 minutes in the conestat alfa 50 units/kg group and 1,101 minutes in the placebo group. The statistical significance of this difference was not tested since a non-significant difference between the higher dose and placebo stopped further testing. [4, 5]

In study C1-1310, time to beginning of symptom relief was defined according to the following responses in the treatment effect questionnaire (TEQ) for the primary attack location:

1. To what extent has the overall severity of your HAE attack changed since you received the infusion? – response of "a little better", "better" or "much better"
2. Overall has the intensity of your HAE attack symptoms begun to decrease noticeably since you received the infusion? – response "yes"
3. Persistence of improvement at the next assessment time point (i.e. same or better response to first question and "yes" to second question.[6]

This was significantly shorter in the conestat alfa group (n=44) compared with placebo (n=31): median time to beginning of symptom relief of 90 minutes and 152 minutes respectively, p=0.031.

The time to minimal symptoms was based on the TEQ question "At this moment, are your HAE attack symptoms minimal (barely noticeable)?" This was numerically, but not significantly, shorter in the conestat alfa group compared with placebo: median of 303 minutes and 483 minutes respectively, p=0.078.[6]

Each controlled study was followed by an open-label extension study during which patients received conestat alfa 50 units/kg (fixed dose of 2,100 units for extension to study C1-1304) for repeated attacks. Patients were treated for up to five attacks and results indicate that the treatment effect, in terms of time to beginning of relief of symptoms, was maintained on repeated dosing.[7, 8]

Summary of evidence on comparative safety

There are no comparative safety data for conestat alfa. Results from placebo-controlled studies for the treatment of a single HAE attack did not highlight any safety differences between conestat alfa and placebo.

There were no cases of thrombotic or anaphylactic adverse events and there were no neutralising antibodies to conestat alfa.

Since conestat alfa is derived from milk of transgenic rabbits and contains traces of rabbit protein, there is a potential risk of hypersensitivity.[1, 2] There is also the potential for development of antibodies, although data available have not suggested that detected antibodies are of clinical relevance.[4]

Summary of clinical effectiveness issues

In each of the controlled studies, the primary outcome was the time to beginning of relief of symptoms and this was significantly reduced with conestat alfa compared with placebo. The reduction was considered by the European Medicines Agency as clinically relevant. Two studies used the VAS score for assessing time to relief from symptoms, while the third study (C1-1310) used the TEQ together with supportive results using the VAS score. It was suggested that the TEQ- based results may be more sensitive to small changes in symptoms as illustrated by the notably shorter time to relief in the placebo group assessed by TEQ compared with VAS score.[6] Two of the studies included a dose of conestat alfa of 100 units/kg which is higher than the licensed dose of 50 units/kg up to a maximum dose of 4,200 units.

In the controlled studies, patients received treatment for one eligible HAE attack. Evidence of repeated treatment from recurrent attacks is available from the open-label extension studies however uncertainties remain as to whether the efficacy would diminish on long term repeated administration in patients who develop antibodies against recombinant C1 esterase inhibitor.[4]

All three controlled studies excluded patients presenting with life-threatening symptoms. This was considered acceptable since the studies included placebo groups but may affect the generalisability of study results to patients with life-threatening symptoms.[4]

Subgroup analyses from studies C1-1205-01 and C1-1304-01 suggested that the treatment effect of conestat alfa was similar for different locations of HAE attacks. However these data are limited by small patient numbers and the inclusion of a higher dose of conestat alfa.[4]

There are no direct or indirect data versus an active comparator and therefore the relative efficacy and safety of conestat alfa is unknown.

The presentation of conestat alfa with solvent and administration kit may allow self-administration for some patients. However, its intravenous route of administration may be a disadvantage for self-injecting. For patients who weigh more than 42kg, two vials need to be reconstituted. [1, 2] Icatibant is available as a solution in pre-filled syringes and is administered subcutaneously.[9] The plasma derived alternatives (Berinert® and Cinryze®) are administered by intravenous injection.[10, 11] The licensed indication for conestat alfa is narrower than for alternatives i.e. for the treatment of acute HAE attacks in adults and adolescents.[1, 2] Therefore potential disadvantages of conestat alfa are that it is not licensed for pre-procedure prevention or routine prevention and that it is not licensed for use in children.

At the PACE meeting, it was noted that conestat alfa provides another C1 inhibitor product which rapidly relieves the symptoms of an acute HAE attack and since it is not plasma derived, the theoretical risk of blood borne infections is avoided giving patients the additional choice of a non-human product.

Patient and clinician engagement

A PACE meeting with patient group representatives and clinical specialists was held to consider the added value of conestat alfa as an ultra-orphan medicine in the context of treatments currently available in NHS Scotland.

The key points expressed by the group were:

- The frequency of HAE attacks can vary significantly between and within individual patients with severity ranging from mild to life threatening. Patients with frequent attacks may be confined to bed or home, while those with infrequent attacks may be affected by the unpredictability in their timing and severity. These situations can be significantly disruptive to normal daily and family living, and to work and education and can have a substantial negative impact on quality of life.

- Conestat alfa provides an additional effective option for the treatment of acute HAE attacks and increases patient choice.

- The availability of an additional C1 inhibitor, including the availability of a non-plasma-derived product, would help mitigate the risk of potential supply shortages which have occurred with other plasma derived products eg immunoglobulins. Any shortage in supplies of C1 inhibitors could put patient lives at risk.

- The currently available C1 inhibitors (Berinert® and Cinryze®) are plasma-derived products and conestat alfa would offer an alternative animal-derived product for patients who wish to avoid plasma-derived products. This could be for religious reasons or to avoid the theoretical risk of human blood borne pathogens. Although no case of infection transmission has been reported to date, the availability of conestat alfa would offer patients an alternative choice.

- The shelf life of conestat alfa is longer than for other available C1 inhibitors and this may be an advantage where supplies are held in remote centres. It may also be an advantage for patients with infrequent attacks who keep a supply of C1 inhibitor at home for emergency use or for travel.

Additional Patient and Carer Involvement

We received a patient group submission from HAEUK, which is a registered charity. HAEUK has received 85% pharmaceutical company funding in the past two years, with none from the submitting company. Representatives from HAEUK participated in the PACE meeting. The key points of their submission have been included in the full PACE statement considered by SMC.

Value for money

The submitting company presented a cost- minimisation analysis of conestat alfa for the treatment of acute angioedema attacks in adults and adolescents with hereditary angioedema (HAE) due to C1 esterase inhibitor deficiency. The comparator was human C1 esterase inhibitor (Berinert®). The time horizon of the model was 72 hours.

In the absence of direct study evidence or a formal indirect comparison, the cost-minimisation analysis used an assumption of equal efficacy between treatments. In the analysis, patients received either a conestat alfa dose of 2,100 units per vial and dosing requirements of 50 units per patient kg, equating to 1 vial per 42kg, or a human C1 esterase inhibitor dose of 500 units per vial with dosing requirements of 20 units per patient kg, which equates to 1 vial per 25kg. Costs associated with treatment included the medicines costs based on an average patient weight of 76.9kg (varied in a scenario analysis to 75kg), and the costs of administration which was administered in hospital for 25% of patients in both groups and at home for 75% of patients in both groups.

Of those receiving their treatment at home, 30% of patients were assumed to be able to self-inject. For the remaining 70%, treatment was costed as being administered by a community nurse. It should be noted that the cost of home administration for patients receiving conestat alfa was assumed to be borne by the submitting company. Of those receiving their treatment in hospital, it was assumed that patients were not admitted but outpatient appointments were costed following the attack. Re-dosing rates were assumed to differ between treatment groups (9.3% for conestat alfa and 18.6% for human C1 esterase inhibitor), but the proportion of patients requiring a re-dose was varied in a scenario analysis. No more than one re-dose was permitted per attack. No adverse event costs were assumed.

A Patient Access Scheme (PAS) was proposed by the submitting company and was assessed by the Patient Access Scheme Assessment Group (PASAG) as acceptable for implementation in NHS Scotland on the list price of the medicine.

The results of the base case and scenario analyses are presented in table one. SMC would wish to present the with-PAS cost-effectiveness estimates that informed the SMC decision but as the PAS is commercial in confidence only the without-PAS figures can be presented.

Table 1: cost-minimisation analysis results versus Human C1 esterase inhibitor (Berinert®), without PAS

Analysis	Type of cost	Conestat alfa (RUCONEST®)	Human C1 esterase inhibitor (Berinert®)	Incremental cost of conestat alfa (RUCONEST®)
Base case	Medicine cost	£1,639.53	£2,217.82	-£578.29
	Administration cost	£26.70	£134.11	-£107.41
	TOTAL	£1,666.24	£2,351.93	-£685.59
Scenario analysis (average weight 75kg not 76.9kg)	Medicine cost	£1,639.53	£1,663.37	-£23.86
	Administration cost	£26.70	£134.11	-£107.41
	TOTAL	£1,666.23	£1,797.47	-£131.27
Scenario analysis (alternative re-dosing rates set at 9.3% for both treatments and 75kg patient weight)	Medicine cost	£1,639.53	£1,532.93	£106.60
	Administration cost	£26.70	£125.69	-£98.99
	TOTAL	£1,666.23	£1,658.62	£7.61
Scenario analysis (All patients at home can self-inject and 75kg patient weight) No PAS	Medicine cost	£1,639.53	£1,6637.37	-£23.86
	Administration cost	£26.70	£26.70	£0
	TOTAL	£1,666.23	£1690.07	-£23.86

*a negative sign in the final column indicates the treatment is cost-minimising against the comparator

The key limitations of the economic evaluation are as follows:

- As noted above, there was no direct or indirect evidence comparing conestat alfa to other relevant treatment options and thus similarity of outcomes was assumed within the cost-minimisation analysis.
- The base case analysis was conducted against a single comparator, despite the availability of another plasma-derived C1 inhibitor (Cinryze®) and existing SMC advice accepting an alternative comparator (icatibant) for use for the same indication. In response to this issue, the company provided some additional analysis to compare conestat alfa to both C1 inhibitor (Cinryze®) and icatibant, assuming rates of re-dosing of 65.7% and 17.8%

respectively for these comparators. Without the PAS, the results showed conestat alfa was associated with cost-savings of £724 and £111 respectively. A potentially more plausible scenario, using re-dosing rates equal to those assumed for conestat alfa, led to a cost increase of £3 and a reduced cost saving of £56 versus each comparator respectively. It should be noted that these figures do not take into account the PAS for icatibant, but results using an estimate of this discount were provided to SMC for use in its decision-making. SMC is unable to present the results provided by the company which used an estimate of the PAS price for icatibant due to commercial confidentiality and competition law issues.

- There was some uncertainty regarding a number of the assumptions used in the analysis, such as the relative rates of re-dosing and the assumptions regarding patients who receive therapy at home. A range of additional sensitivity analyses were provided, and selected key results shown in table 1 above.

*Other data were also assessed but remain commercially confidential.**

Impact beyond direct health benefits and on specialist services

The PACE participants noted that plurality of available C1 inhibitors, including a non-plasma product, would help mitigate the risk of supply shortages which have occurred with other plasma-derived products. Any shortage in supplies of C1 inhibitors could put patient lives at risk. The shelf life of conestat alfa is longer than for other available C1 inhibitors which may be an advantage for patients with infrequent attacks who keep a home supply for emergency use or for travel. It may also be an advantage where supplies are held in remote centres.

Costs to NHS and Personal Social Services

The submitting company estimated there would be 102 patients eligible for treatment with conestat alfa in all years. The estimated uptake rate was 4% in year 1 (4 patients) rising to 15% in year 5 (15 patients).

SMC is unable to publish the with PAS budget impact due to commercial in confidence issues. A budget impact template is provided in confidence to NHS health boards to enable them to estimate the predicted budget with the PAS.

The submitting company did not estimate any costs outside the NHS.

*Other data were also assessed but remain commercially confidential.**

Conclusion

The Committee considered the benefits of conestat alfa in the context of the SMC decision modifiers that can be applied and agreed that as conestat alfa is an ultra-orphan medicine, SMC can accept greater uncertainty in the economic case.

After considering all the available evidence, output from the PACE process, and after application of the appropriate SMC modifiers, the Committee accepted conestat alfa for use in NHS Scotland.

Additional information: guidelines and protocols

In 2015 a UK consensus guideline commissioned by the Primary Immunodeficiency Network (PIN) and the patient group Hereditary Angioedema UK (HAE UK) was published "CI inhibitor defiency: 2014 consensus document".[3] For acute treatment, this states that plasma-derived C1 inhibitors (Berinert®, Cinryze®), recombinant C1 inhibitor (Ruconest®) and icatibant (Firazyr®) are all acceptable options. Icatibant may be particularly useful in enabling self-administration as intravenous access is not necessary. It also notes that plasma-derived C1 inhibitors is the treatment of choice for acute attacks of HAE for children, pregnant and breast-feeding women and those trying to conceive.
This guidance predates the availability of the self-administration presentation of conestat alfa.

In 2012, the World Allery Organisation (WAO) published "WAO guideline for the management of hereditary angioedema".[12] The guideline recommends that on-demand treatment should be considered for all attacks that result in debilitation/dysfunction and/or involve the face, neck or abdomen. Treatment is mandatory for attacks affecting the upper airways. Attacks should be treated as early as possible with C1 esterase inhibitor, icatibant or ecallantide (not licensed in the UK). If none of the recommended first-line therapies are available, the use of solvent detergent-treated plasma, or frozen plasma could be considered.

Additional information: comparators

Plasma-derived C1 esterase inhibitors (Berinert® and Cinryze®) or icatibant.

Cost of relevant comparators

Medicine	Dose Regimen	Cost per attack (£)
Conestat alfa	**50 units/kg body weight intravenous injection (patients weighing ≥84kg, 4,200 units)**	**1,500 (to 3,000)***
Human C1-esterase inhibitor (Berinert®)	20 units/kg body weight intravenous injection	1,402
Icatibant	30mg subcutaneous injection, repeated to a maximum of three doses in 24 hours.	1,395 (to 4,185)**
Human C1-esterase inhibitor (Cinryze®)	1,000 units, repeated after 60 minutes if inadequate response	1,336 to 2,672

*Doses are for general comparison and do not imply therapeutic equivalence. Costs from eMIMS on 3 April 2018. Costs calculated using the full cost of vials/ampoules assuming wastage. Weight based doses calculated from an adult bodyweight of 70kg. * the SPC for conestat alfa states that in the majority of cases a single dose is sufficient to treat an acute attack but an additional dose can be given if there is an insufficient clinical response. ** the SPC for icatibant states that in the majority of cases a single injection is sufficient to treat an attack but that a second and third injection can be given if there is insufficient relief or recurrence of symptoms. Costs do not take any patient access schemes into consideration.*

References

1. Pharming Group NV. Conestat alfa (Ruconest) 2100U powder and solvent for solution for injection, Summary of Product Characteristics. Last updated 9 October 2017.
2. Pharming Group NV. Conestat alfa (Ruconest) 2100U powder for solution for injection, Summary of Product Characteristics. Last updated 9 October 2017.
3. Longhurst HJ, Tarzi MD, Ashworth F, Bethune C, Cale C, Dempster J*, et al.* C1 inhibitor deficiency: 2014 United Kingdom consensus document. Clinical and Experimental Immunology. 2015;180(3):475-83.
4. European Medicines Agency. RUCONEST: EPAR Public assessment report. Available at: http://www.ema.europa.eu/docs/en_GB/document_library/EPAR_-_Public_assessment_report/human/001223/WC500098546.pdf. 2010:57.
5. Zuraw B, Cicardi M, Levy RJ et al. Recombinant human C1-inhibitor for the treatment of acute angioedema attacks in patients with hereditary angioedema. J Allergy Clin Immunol 2010;126:821-827.
6. Riedl MA BJ, Li H et al. Recombinant human C1-esterase inhibitor relieves symptoms of hereditary angioedema attacks: Phase 3, randomized, placebo-controlled trial. Annals of Allergy, Asthma and Immunology. 2014;112:163-9.e1.
7. Riedl MA, Levy RJ, Suez D, Lockey RF, Baker JW, Relan A*, et al.* Efficacy and safety of recombinant C1 inhibitor for the treatment of hereditary angioedema attacks: a North American open-label study. Ann Allergy Asthma Immunol; 2013
8. Li HH, Moldovan D, Bernstein JA, Reshef A, Porebski G, Stobiecki M*, et al.* Recombinant Human-C1 Inhibitor Is Effective and Safe for Repeat Hereditary Angioedema Attacks. Journal of Allergy and Clinical Immunology: In Practice. 2015;3(3):417-23.
9. Shire Human Genetic Therapies. Icatibant (Firazyr) 30mg solution for injection in pre-filled syringe, Summary of Product Characteristics. Last updated 30 October 2017.
10. CSL Behring UK Ltd. Berinert 500IU and 1500IU powder and solvent for solution for injection, Summary of Product Characteristics. Last updated 21 November 2017.
11. Shire Pharmaceutical Ltd. Cinryze 500IU powder and solvent for solution for injection, Summary of Product Characteristics. Last updated 29 September 2017.
12. Craig T A-PE, Bork K et al. WAO Guideline for the Management of Hereditary Angioedema. The World Allergy Organization journal
World Allergy Organ J; 2012

This assessment is based on data submitted by the applicant company up to and including 15 June 2018.

**Agreement between the Association of the British Pharmaceutical Industry (ABPI) and the SMC on guidelines for the release of company data into the public domain during a health technology appraisal: http://www.scottishmedicines.org.uk/About_SMC/Policy_statements/Policy_Statements*

Medicine prices are those available at the time the papers were issued to SMC for consideration. SMC is aware that for some hospital-only products national or local contracts may be in place for comparator products that can significantly reduce the acquisition cost to Health Boards. These contract prices are commercial in confidence and cannot be put in the public domain, including via the SMC Detailed Advice Document. Area Drug and Therapeutics Committees and NHS Boards are therefore asked to consider contract pricing when reviewing advice on medicines accepted by SMC.

Patient access schemes: A patient access scheme is a scheme proposed by a pharmaceutical company in order to improve the cost-effectiveness of a medicine and enable patients to receive access to cost-effective innovative medicines. A Patient Access Scheme Assessment Group (PASAG), established under the auspices of NHS National Services Scotland reviews and advises NHS Scotland on the feasibility of proposed schemes for implementation. The PASAG operates separately from SMC in order to maintain the integrity and independence of the assessment process of the SMC. When SMC accepts a medicine for use in NHS Scotland on the basis of a patient access scheme that has been considered feasible by PASAG, a set of guidance notes on the operation of the scheme will be circulated to Area Drug and Therapeutics Committees and NHS Boards prior to publication of SMC advice.

Advice context:

No part of this advice may be used without the whole of the advice being quoted in full.

This advice represents the view of the Scottish Medicines Consortium and was arrived at after careful consideration and evaluation of the available evidence. It is provided to inform the considerations of Area Drug & Therapeutics Committees and NHS Boards in Scotland in determining medicines for local use or local formulary inclusion. This advice does not override the individual responsibility of health professionals to make decisions in the exercise of their clinical judgement in the circumstances of the individual patient, in consultation with the patient and/or guardian or carer.