Patient Group Submission Form

The Scottish Medicines Consortium (SMC) is committed to working in partnership with patient groups to capture patient and carer experiences, and use them to inform decision-making.

Before you make a submission

You are required to complete a Patient Group Partner Registration Form before you make a submission. The registration form requests general information about your organisation. It only needs to be completed once (and annually updated) and should save you time with any further submissions to SMC. If you have not already completed a registration form, please do this before you make your submission.

You will find it helpful to read our Guide for Patient Group Partners, which gives details about the type of information you need to capture in the submission form. **Please read this before you make your submission and use it to help you complete each question.**

You can find the Registration Form and Guide for Patient Group Partners here:

https://www.scottishmedicines.org.uk/Public_Involvement/Submission_form_and_guidance

Contact us

If you have any more questions after reading the guide, the SMC Public Involvement Team can support you throughout the submission process. You can email us at: hcis.SMCPublicInvolvement@nhs.net or phone: **0141 414 2403.** Please do not hesitate to get in touch, as we are here to help you.

Name of medicine:

RoActemra (Tocilizumab)

Indication: (what the medicine is used for)

Giant Cell Arteritis

Submission date:

24th May 2018

Name of organisation making submission:

Polymyalgia Rheumatica and Giant Cell Arteritis Scotland (PMR-GCA Scotland)

Who is the main contact for submissions to SMC?

Name:	Lorna Neill
Position held in organisation:	Chair
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Summary of Key Points

Please summarise the key points of your submission which you would like to emphasise to SMC Committee – bullet points may be helpful.

(See P11 of A Guide for Patient Group Partners)

300 words maximum

GCA affects older people many of whom are already struggling to cope with other illnesses and disabilities. It is twice as common in women as men, with a lifetime risk of 1.0% cf. 0.5% in those over 50.

Often neither the patients nor family and friends have heard of the illness and few GPs have much experience of it. Treatment is often suboptimal or delayed and too many still lose vision unnecessarily.

Prednisolone treatment in high doses quickly reduces inflammation and protects against sight loss but can be very difficult to tolerate.

At the high doses used initially, steroid psychosis or just anxiety and hyperactivity is frightening, causing some to reduce too fast or resist increasing their dose if a subsequent flare occurs. Yo-yo effects in dosage then lead to poor control of symptoms and increased time on prednisolone.

Overwhelming fatigue, often a feature of auto-immune illnesses, is unfortunately not improved by prednisolone and many have the pain and stiffness of PMR in addition.

Side effects are common and serious (diabetes, osteoporosis, high blood pressure, cataract and susceptibility to infections) and add to the burdens of increasing age.

While some achieve complete remission of symptoms and an uneventful taper to zero prednisolone within two years, for most of those who contact the charity this has not happened. They still have polymyalgic symptoms and frightening flares with return of head pains and jaw stiffness after many years of treatment (17years in one case) and expect to remain on prednisolone, increasing their cumulative dose and risk of serious adverse effects, for life.

A medication more likely to achieve symptom- free remission, shorten treatment time and reduce

steroid side effects would give this group their lives back at a time when the number of quality years remaining to them is especially precious.

Please provide details of any individuals who have had a significant role in preparing your submission and who have an interest to declare.

(See P11 of A Guide for Patient Group Partners)

300 words maximum

No individual involved has any interest to declare

Please tell us how you gathered information about the experiences of patients and carers to help inform your submission.

(See P11 of A Guide for Patient Group Partners)

300 words maximum

Discussions took place within support groups in Dundee, Glasgow, Edinburgh and Inverness.

Notes were taken from Helpline calls and emails to the charity, describing members' experiences and their requests for help.

Questionnaires were given out at our AGM, made available on the website and sent to some members known to have GCA. There were 14 written responses from members with GCA . The poor response to email or web questionnaires reflects the age range of our members and the few for whom we hold email addresses.

1. How does this condition affect the day-to-day lives of people living with it? (See P11 of A Guide for Patient Group Partners)

500 words maximum

Our members complained initially of feeling ill, feverish or with symptoms of extreme cold, loss of appetite, weight loss, depression, severe headache and scalp tenderness so bad it was difficult to lie on a pillow. Some had jaw pain and stiffness on chewing, some double vision and others episodes of temporary sight loss. A few unfortunates suddenly lost vision permanently. For them, learning to cope afterwards was hard and many never felt confident to drive again. Even unilateral sight loss was problematic if cataract and macular degeneration had already damaged sight in the other eye. Previously diagnosed polymyalgia rheumatica was common while many developed symptoms of this after diagnosis.

Once on treatment with high dose steroids anxiety levels are high partly due to the steroids themselves but also the fear of missing a recurrence and losing (more) vision. This is accompanied by complaints of poor memory "difficulty with planning " and frustration at being unable to cope with simple tasks "like drying my hair, bending, lifting, getting in and out of a car".

Mobility problems due to pain and stiffness in shoulders and hips, dizziness and shaking, make everyday tasks an effort and falls frequent. Walking aids and a mobility badge can be required.

For elderly women in particular who are carers for even older husbands, if they are unable to cope with housework and cooking both may have to go into care.

Almost without exception there are complaints of incapacitating tiredness which persisted even after treatment. Many need to sleep for two hours in the afternoon. One member described her feelings of "every day having the worst flu" and being unable to plan activities or enjoy the social life she had had previously. Another said "I can now do only one thing a day - something am OR pm but NEVER in the evening. I no longer entertain". Such admissions are often accompanied by feelings of guilt that family members are also missing out, especially for those at the younger end of the age range who had planned an active retirement with a partner or could no longer undertake caring duties for grandchildren.

"I felt miserable, upset, angry, helpless, useless, in despair sometimes that I would not get better. At

times my husband felt like my carer which made me feel inadequate."

Family members vary in their ability to understand or empathise from "a totally understanding husband who has been fully supportive throughout " to "They think I am a hypochondriac".

Those who are still working find this constant fatigue plus difficulty caused by morning stiffness and "brain fog" make continuation of their previous employment impossible. A nurse, diagnosed at 57, was "off sick for ten months, had a phased return but had to leave nursing because of continuing ill health due to GCA". Even an initially sympathetic employer finds the duration of the illness and frequent relapses, especially during the first two years, difficult to accommodate. As retirement age has risen, this problem will increase.

2. How well do medicines which are currently available in NHSScotland help patients manage this condition? (See P12 of A Guide for Patient Group Partners)

500 words maximum

High dose glucocorticoids (usually prednisolone) are the only treatment currently offered initially, given by infusion in hospital if considered an emergency, or taken as a daily dose of tablets first thing in the morning. Some split this trying to control return of symptoms in the evening or avoid disturbed sleep. The steroids control severe inflammation quickly and protect against further sight loss, removing the pain from head and jaw and the symptoms of PMR. At this stage however they can bring severe psychiatric symptoms described by one male patient as "feelings of god-like invincibility". Another member described being "quite manic – spending a lot of money and high activity with very little sleep alternating with periods of extreme fatigue." Others become anxious and depressed especially as symptoms do not always disappear completely, just reduce to manageable levels.

Along with prednisolone, patients will usually be prescribed bone protection, eg alendronic acid, Ca and vitamin D. Most will get a PPI for gastric protection and, if relapses are frequent or there is difficulty reducing the prednisolone dose some will be offered a DMARD, most often methotrexate, in addition.

All of the above come with side effects and while not all patients get them all, some have great problems managing these along with other medications for co-existing conditions, common in this age group, such as cardiovascular disease, diabetes, thyroid treatment and arthritis (RA and OA).

The problem long term becomes managing the steroid reduction schedule. This was reported, in a survey by our sister charity (PMRGCAuk), as the second most difficult thing to deal with after the illness itself. Medical professionals emphasise the need to be on the lowest dose possible but the underlying illness does not go away, so each month a reduction is made and the patient must decide over a week or so whether any return of symptoms is temporary, is worsening due to now being on too low a dose or whether the disease is flaring badly and will need a return to a much higher dose again. This can cause great anxiety and be demoralising when a lower dose has taken many months to achieve.

At high to medium doses few avoid the side effects of weight gain around the midriff, across shoulders and on cheeks. Women in particular experience their changed appearance as distressing

especially if it includes hair growth on the face along with loss of hair on the head. More serious and lasting side effects are osteoporosis, sometimes with fracture, diabetes, glaucoma, high blood pressure and cataracts along with increased susceptibility to infections, especially UTIs .

Regular monthly blood tests for inflammatory markers can help some manage their reductions but for others these do not correlate well with symptoms, leading to friction with medical professionals as to whether the dose should be reduced. One helpline phone call was made by a gentleman in his eighties, in tears because he could not get out his bed after following his GP's instructions for a large reduction.

3. Have you been able to consult with patients who have used this medicine? (See P12 of A Guide for Patient Group Partners)

Yes ☐ **No** ☒

4. Would this medicine be expected to improve the patient's quality of life and experience of care, and if so, how?

(See P12 of A Guide for Patient Group Partners)

500 words maximum

As we had no reports from members using the drug we can only respond to the claims made subsequent to the GiACTA trial:-

Sustained remission in one year would be so much better than what is currently the norm i.e. about three years of treatment for the lucky ones who can taper at near the suggested rate and longer, sometimes many years longer, for many. A proportion of patients at present never feel themselves to be in remission and always have some level of symptoms. It is common for there to be a problem reducing at the suggested 1mg/month when 7-8mg is reached and the last few mg are often only achieved by using what patient organisations call the "dead slow, nearly stop" method. In practice therefore few reduce to zero prednisolone within the two years often quoted at first diagnosis, even without having had severe flares.

When questioned about relapses or flares, twice as many members reported having relapsed as did not. This leads to more frequent visits to GPs and hospital consultants for blood tests and sometimes scans. A sustained period of being symptom free would reduce anxiety in patients and would allow them to normalise their lives sooner than at present. "I felt like I lost 2 years of my life." Younger patients who are still working could hope to return sooner.

A smaller cumulative steroid dose should reduce the incidence of adverse effects like the development of osteoporosis, cataract or diabetes, greatly decreasing the patients' need for medication and hospital visits. "Going to the GP was fine as in a nearby village. Going to hospital appointments – rheumatology, haematology and opthalmology was a 20mile journey to Glasgow and very stressful." By reaching a low dose of prednisolone sooner the less serious but still

distressing side effects of weight gain and hair loss may be brought under control more easily, improving self esteem and encouraging physical activity and social interaction.

Patients have frequently reported that they are prepared to accept some side effects and long-term risk if they can improve their current quality of life because having the untreated illness reduces this to unacceptable levels, as well as putting them at risk of sight loss and stroke. A member who developed GCA with PMR in 2001 and took steroids for 15 years said, "Reducing the prednisolone dose quickly was not a priority, although both my Achilles tendons and my neck became severely weakened in 2016 - but I had NO REGRETs (her emphasis). I had been able to do what I wanted to do and had the quality of life I needed during the preceding years." Shortening the period of time till remission might well have avoided these adverse effects of treatment which was necessary for her to maintain an active life.

For those who can not take steroids for medical reasons, who suffer severe mental or physical side effects or continue to have overwhelming fatigue during treatment, tocilizumab offers hope.

5. What kind of impact would treating a patient with this medicine have on the patient's family or carers? (See P13 of A Guide for Patient Group Partners)

500 words maximum

Shortening the period of illness, especially that on high doses of steroids which is associated with mania or depression, and reducing flares and the anxiety caused by returning symptoms, will reduce stress on those who care for patients with GCA.

Many patients are also carers for older family members or for younger grandchildren. They should be able to return to these roles sooner, benefiting all the family.

For those many patients who can not at present be brought into remission by prednisolone alone this could afford all family members a return to normal life.

6. Are there any disadvantages of the new medicine compared to current standard treatments? (See P13 of A Guide for Patient Group Partners)

500 words maximum

A weekly injection may be disliked, especially by elderly patients and some carers may be unwilling or unable to give it. This would mean more visits to the GP, although we note that Roche provide a homecare service which could solve this problem.

7. Is there any additional information you think may be useful for the SMC committee to consider? (Optional)

500 words maximum

8. Do you consent for a summary of your submission to be included in the Detailed Advice Document for this medicine?

Yes ☒ No ☐

Thank you for completing this form.

Please email it to: hcis.SMCPublicInvolvement@nhs.net

If you are unable to email this form to us, please send by post to the address below:

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The Public Involvement Team is available to advise you on how to complete this form to ensure the patient and carer experience is fully captured, to help inform the SMC decision making process. If you have any questions about completing this form call us on: 0141 414 2403.