NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Patient/carer organisation submission (STA)

Edoxaban tosylate for preventing stroke and systemic embolism in people with non-valvular atrial fibrillation

Thank you for agreeing to give us your views on this treatment that is being appraised by NICE and how it could be used in the NHS. Patients, carers and patient organisations can provide a unique perspective on conditions and their treatment that is not typically available from other sources. We are interested in hearing about:

- the experience of having the condition or caring for someone with the condition
- the experience of receiving NHS care for the condition
- the experience of having specific treatments for the condition
- the outcomes of treatment that are important to patients or carers (which might differ from those measured in clinical studies, and including health-related quality of life)
- the acceptability of different treatments and how they are given
- expectations about the risks and benefits of the treatment.

To help you give your views, we have provided a questionnaire. You do not have to answer every question — the questions are there as prompts to guide you. The length of your response should not normally exceed 10 pages.

1. About you and your organisation

Your name:

Name of your organisation: AntiCoagulation Europe

Your position in the organisation:

Brief description of the organisation:

(For example: who funds the organisation? How many members does the organisation have?)

Independent charity – aims are the prevention of thrombosis, provision of information and support to people taking anticoagulant or antiplatelet therapies for a range of conditions – VTE, Atrial Fibrillation, mechanical valve replacement and thrombotic disorders.
Membership is supported by subscriptions and resources include a website www.anticoagulationeurope.org, a quarterly publication INreview and a dedicated helpline for patients, carers and healthcare providers. We are committed to striving for continuous improvements in the delivery of patient centric anticoagulant services across all areas of healthcare.

We are asking for your collective view as an organisation and will be asking patient experts for their individual input separately. If you have the condition, or care for someone with the condition, you may wish to complete a patient expert questionnaire to give your individual views as well.

2. Living with the condition

What is it like to live with the condition or what do carers experience when caring for someone with the condition?

Atrial Fibrillation describes a heart rhythm disturbance which can cause a person to experience a highly irregular pulse rate. When blood fails to be pumped effectively by the heart mechanism, blood can pool in the atria and clots can form which can then travel through the circulatory system and if it reaches the brain, can cause a stroke. AF can be intermittent, coming and going without any warning and sometimes with long periods between

episodes. Some people don't even realise they have AF until it is picked up whilst they are being tested for other conditions. Some episodes last less than 7 days and may not require treatment (paroxysmal), persistent AF may last 7 days or less when treated and permanent or longstanding persistent AF is continous AF which has occurred for more than one year.
Once AF is diagnosed, it is important for the individual to be given anticoagulant treatment to reduce the risk of stroke. Other medications to help control the irregular heartbeat will be prescribed and in some cases surgical interventions may be offered to help correct the heart rhythm. AF risk increases with age – 1 in 10 people will get AF over the age of 65 and AF has a five fold risk of stroke which can impact on communication, mobility, vision and cognitive ability. Symptoms can include palpitations (thumping heart) dizziness, chest pains and breathlessness. When experiencing an episode, it can cause anxiety and distress to the person or carer. In some circumstances, people can experience a Transient ischaemic attack (TIA) or mini stroke when a small blood clot gets stuck in the artery of the brain affecting blood flow and causing symptoms such as visual disturbance, numbness, weakeness in the arms and legs. These effects do not last as long as a stroke but can cause stress to the individual. AF sufferers need to protect against stroke risk by taking oral anticoagulants such as warfarin (VKA) which needs regular monitoring or one of the newer agents (NOACS) as recommended by their managing clinician.

Which treatment outcomes are important to patients or carers? (That is, what would patients or carers like treatment to achieve?) Which of these are most important? If possible, please explain why.

Efficacy and Safety - prevention and protection against the risk of a debilitating stroke which could impact on the health and mental well – being of the individual, family and carers.

Financial implications of not being able to work or having to arrange for care to support the individual.

Convenience. Warfarin requires regular monitoring with blood tests to check INR levels are in therapeutic range. People need to attend anticoagulation clinics in secondary or primary care often on a regular basis. Warfarin needs to be dose adjusted and can be affected by diet and interacts with other drugs.

What is your organisation's experience of currently available NHS care and of specific treatments for the condition? How acceptable are these treatments and which are preferred and why?

NCE Guidelines recommends the following anticoagulants for AF:

Warfarin - oral anticoagulant. Dose adjustment required to keep patient in therapeutic range. Monitoring by regular blood tests. Some people may self – test using a hand held device. Some patients may undertake self management testing and dosing with the consent of their doctor

Dabigatran – new oral agent. Twice daily dose, no monitoring required

Rivaroxaban – new oral agent Once daily dose, no monitoring required

Apixaban - new oral agent. Twice daily dose, no monitoring required

Dabigatran, Rivaroxaban and Apixaban are not affected by diet and do not appear to interact with other medications as extensively as warfarin. To date, there is no antidote available for the newer agents.

3. What do patients or carers consider to be the advantages of the treatment being appraised?

Benefits of a treatment might include its effect on:

- the course and/or outcome of the condition
- physical symptoms
- pain
- level of disability
- mental health
- quality of life (such as lifestyle and work)
- other people (for example, family, friends and employers)

- ease of use (for example, tablets rather than injection)
- where the treatment has to be used (for example, at home rather than in hospital)
- any other issues not listed above

Please list the benefits that patients or carers expect to gain from using the treatment being appraised.

This treatment does not yet appear to be licenced for use in the UK. It gained FDA approval in USA for treatment of nonvalvular AF in Jan 2015.

ACE has reviewed the results of the Engage – TIMI 48 trial Edoxaban versus Warfarin in Patients with Atrial Fibrillation; Giugliano .R.P. et al as published in N Engl J Med 2013; 369:2093-2104November 28, 2013 DOI: We10.1056/NEJMoa1310907.

We note that the study concluded ‘ that both once-daily regimes of edoxaban were non – inferior to warfarin for the prevention of stroke or systemic embolism and were associated with significantly lower rates of bleeding and death from cardiovascular causes.’ ACE is unable to comment on how this will affect patients or carers at this stage.

Please explain any advantages that patients or carers think this treatment has over other NHS treatments in England.

Unable to comment as no marketing licence in place in UK

If you know of any differences in opinion between patients or carers about the benefits of the treatment being appraised, please tell us about them.

N/A

4. What do patients and/or carers consider to be the disadvantages of the treatment being appraised?

Disadvantages of a treatment might include:

- aspects of the condition that the treatment cannot help with or might make worse
- difficulties in taking or using the treatment (for example, injection rather than tablets)
- side effects (for example, type or number of problems, how often, for how long, how severe. Please describe which side effects patients might be willing to accept or tolerate and which would be difficult to accept or tolerate)

- where the treatment has to be used (for example, in hospital rather than at home)
- impact on others (for example, family, friends and employers)
- financial impact on the patient and/or their family (for example, the cost of travel to hospital or paying a carer)
- any other issues not listed above

Please list any concerns patients or carers have about current NHS treatments in England.

As with any of the new anticoagulants, there is no known antidote.

Access to NOACS is slow in the UK and approx 50% of the AF population at risk are not being anticoagulated with either warfarin or one of the newer approved agents. NICE AF guidelines were updated in 2014 to reflect recommendations of Dabigatran, Rivaroxaban and Apixaban being treatments alongside warfarin for AF patients assessed at risk of stroke.

Please list any concerns patients or carers have about the treatment being appraised.

Not yet licenced for UK market.

If you know of any differences in opinion between patients or carers about the disadvantages of the treatment being appraised, please tell us about them.

N/A

5. Patient population

Are there any groups of patients who might benefit more from the treatment than others? If so, please describe them and explain why.

If marketing licence obtained and NICE recommends, further treatment option for AF population.

Are there any groups of patients who might benefit less from the treatment than others? If so, please describe them and explain why.

We note that the FDA makes reference to the need to assess kidney function prior to treatment with dosing regime being advised dependent on individual patient's results.

Research evidence on patient or carer views of the treatment

Is your organisation familiar with the published research literature for the treatment?

☐x Yes ☐ No

If you answered 'no', please skip the rest of section 7 and move on to section 8.

Please comment on whether patients' experience of using the treatment as part of their routine NHS care reflects the experiences of patients in the clinical trials.

N/A

Do you think the clinical trials have captured outcomes that are important to patients? Are you aware of any limitations in how the treatment has been assessed in clinical trials?

No comment

If the treatment being appraised is already available in the NHS, are there any side effects that were not apparent in the clinical trials but have emerged during routine NHS care?

N/A

Are you aware of any relevant research on patient or carer views of the condition or existing treatments (for example, qualitative studies, surveys and polls)?

☐ Yes ☐x No

If yes, please provide references to the relevant studies.

6. Equality

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Protected characteristics are: age; being or becoming a transsexual person; being married or in a civil partnership; being pregnant or having a child; disability; race including colour, nationality, ethnic or national origin; religion, belief or lack of religion/belief; sex; sexual orientation.

Please let us know if you think that recommendations from this appraisal could have an adverse impact on any particular groups of people, such as:

- excluding from full consideration any people protected by the equality legislation who fall within the patient population for which the treatment is/will be licensed;
- having a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the treatment;
- any adverse impact on people with a particular disability or disabilities.

Please let us know if you think that there are any potential equality issues that should be considered in this appraisal.

None at this stage

Are there groups of patients who would have difficulties using the treatment or currently available treatments? Please tell us what evidence you think would help the Committee to identify and consider such impacts.

Unable to comment - no marketing licence available at this stage

7. Other issues

Do you consider the treatment to be innovative?

☐ Yes ☐x No

If yes, please explain what makes it significantly different from other treatments for the condition.

Are there any other issues that you would like the Appraisal Committee to consider?

None at this stage

8. Key messages

In no more than 5 bullet points, please summarise the key messages of your submission.

- No marketing licence available to date
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