

Draft Patient Expert Submission Template

Thank you for agreeing to give us a personal statement on your view of the technology and the way it should be used in the NHS.

Patients and patient advocates can provide a unique perspective on what they would like from a technology, which is not typically available from the published literature.

To help you in making your statement we have provided a template. The questions are there as prompts to guide you. You do not have to answer every question. A short, focused reply, giving a patient's perspective, is what we need. Your statement can be as short as you like, and we suggest a maximum of 4 pages.

What do patients and/or carers consider to be the advantages and disadvantages of the technology for the condition?

1. Advantages

(a) Please list any aspects of the condition that you expect the technology to help with. For each aspect you list please describe, if possible, what difference you expect the technology to make.

Myeloma is a complex and debilitating cancer which is currently incurable. Patients living with myeloma commonly experience a number of debilitating complications associated with the disease.

These include, but are not limited to, severe bone destruction accompanied by severe pain, recurrent infections, renal impairment, bleeding disorders, anaemia and nervous complications.

After decades of limited development and a poor outlook, the last 5 years or so have seen quite a significant number of very positive developments. While a 'silver bullet' treatment and cure may still elude us for years to come there is now real hope that myeloma can become a disease that people live with rather than die from.

Velcade is an important and integral part of this reality for the following reasons.

- It provides an effective, tolerable and evidence based option for patients with relapsing disease after either one or more previous treatment options. No current evidence based standard of care currently exists.
- Velcade has been shown to improve outcomes in myeloma patients and can extend survival
- Responding patients will benefit from an improvement in the debilitating symptoms of myeloma including stabilisation or improvement of their renal disease, bone disease, blood counts etc.
- This results in an overall improvement in quality of life which is such an important factor for patients living with a terminal and debilitating cancer.

- Velcade is not a last option for myeloma patients but can provide the platform from which patients can benefit from newer drugs such as Revlimid and soon to be available pipeline treatments such as Tubacin and Heat Shock Protein-90 Inhibitors

Myeloma has multiple mechanisms of action and pathways and the need for new classes of drugs to be used in combination with existing and future treatments can not be stressed highly enough

Taken together, Velcade is playing a critical role in improving survival outcomes and quality of life for myeloma patients and in doing so is making an important contribution to the government goal of reducing cancer deaths.

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(b) Please list any short-term and/or long-term benefits that patients expect to gain from using the technology. These might include the effect of the technology on:

- the course and/or outcome of the condition

Although not a curative treatment Velcade offers the realistic prospect of increased survival and the platform from which to use other new treatments alone or in combination.

- physical symptoms

Through reducing a patients myeloma cells, Velcade can benefit patients through improving the debilitating symptoms associated with the disease including stabilisation or improvement of their renal disease, bone disease, blood counts etc. As a result, most patients see a significant improvement in their quality of life

- pain

By treating the underlying problem which is the myeloma, this reduces the activity of the myeloma on the bones of the body and resulting bone disease and therefore an improvement in pain is often see. It is worth noting however that bone already damaged due to myeloma bone disease cannot be repaired and that some degree of pain is a constant presence for the vast majority of people living with myeloma.

- mental health

The hope and positive impact that Velcade offers is almost immeasurable – you cannot imagine how important it is to know that new, effective drugs exist and other options are available.

The biggest negative impact on mental health of both the patient and the carer is living with the tantalising, traumatic, time consuming uncertainty that this treatment will not be available where they live or when they will need it.

- quality of life (lifestyle, work, social functioning etc.)

As stated elsewhere, Velcade has a notable positive impact on quality life even after only moderate responses.

- other people (for example family, friends, employers)

The impact of additional life, improvement in mental state and quality of life of a loved one with myeloma is truly incalculable. The treatment of cancer is not just about a disease, but a person who has a disease who is part of what I call the 'cancer family'

Modest investments of time, resource and finance within the context of cancer treatment can have quite significant benefits at all levels to many people.

2. Disadvantages

Please list any problems with or concerns you have about the technology.

Disadvantages might include:

- aspects of the condition that the technology cannot help with or might make worse (see list in (b) above for suggested items)

Velcade is an effective, beneficial myeloma agent, however, it is by no means a silver bullet, and is not curative. However, due to the nature of myeloma, no available therapies offer a cure.

Side effects are an inevitable consequence of cancer therapies, but the majority of patients find that, on the whole, the side effects are tolerable and can be managed.

- difficulties in taking or using the technology

Velcade is available to most myeloma patients and would only be recommended for use in patients who fit the prescribing criteria.

- side-effects (please describe which side effects patients might be willing to accept or tolerate and which would be difficult to accept or tolerate)

Side effects are an inevitable consequence of cancer therapy. In my experience, the vast majority of patients find the side-effect profile tolerable and acceptable and view these as an acceptable balance for the outcome achieved.

- impact on others (for example family, friends, employers)

There is clearly a potential impact here on various members of the community. However, I don't see the impact that Velcade has as being any different from any other cancer agent.

- financial impact on the patient or their family (for example cost of travel needed to access the technology, or the cost of paying a carer).

As above, there is a cost. Patients are already being forced to pay for this treatment and to sell house, cash in investments and use life savings because the NHS will not pay up. That is what patients are prepared to do. The cost of travel etc for most, while not insignificant, will be entirely acceptable to the vast majority of patients and their families.

3. Are there differences in opinion between patients about the usefulness or otherwise of this technology? If so, please describe them.

None

4. Are there any groups of patients who might benefit **more** from the technology than others? Are there any groups of patients who might benefit **less** from the technology than others?

The patients that do well are the one that can access the treatment when their consultant recommends it for use. It works less well on patients who have to fight for access and in some case have had to wait 7 weeks to receive the treatment. Its use should not be delayed because doctors have to go through nonsensical red tape but be available when patients need it.

The advantages and disadvantages of the technology *compared with current standard practice*

NICE is particularly interested in your views on how the technology compares with current standard practice (alternatives if any) used in the UK.

Please list any alternatives available as far as you are aware in current standard practice to the technology

There is no standard treatment protocol for the treatment of relapsed myeloma and no consensus of opinion exists. Guidelines on the Diagnosis, Treatment and Management of Myeloma have been prepared by the UK Myeloma Forum on behalf of the British Committee for Standards in Haematology, but a patient pathway through treatment will be affected by many disease and patient-specific factors, and patient treatment pathways will differ from hospital to hospital.

Eric Low Submission – Eric Low

Single Technology Appraisal of bortezomib for the treatment of multiple myeloma

Commonly used drugs at this stage of disease include thalidomide either alone or in combination with dexamethasone, melphalan-prednisone and dexamethasone.

(i) If you think that the new technology has any **advantages** for patients over other current standard practice, please describe. Advantages might include:

- improvement in the condition overall or in certain aspects of the condition

Velcade not only offers a significant anti-myeloma effect with 43% of patients achieving a complete or partial response. This is a high response rate for a single agent. Response to therapy is associated with improvement in the debilitating symptoms and can result in a marked improvement in overall quality of life.

Velcade offers a new way of treating myeloma and as a result can be used in patients who no longer respond to standard therapies.

- ease of use (for example tablets rather than injection, at home rather than in hospital)

Although the administration of Velcade is straight forward and only last a matter of seconds, it does require the patient to attend hospital twice a week. While this no doubt may be problematic for some patient, this does not present a problem for the vast majority of patients.

A recent IMF (UK) survey showed that 51% of patients preferred to receive their treatment in the hospital rather than at home (Data on File).

- fewer side effects (for example number of problems, frequency, duration, severity)

On the whole, Velcade is a well tolerated treatment. Most patients report very few symptoms and for those that do, peripheral neuropathy is the biggest problem.

It is not clear however how much of this peripheral neuropathy is due to previous treatments such as vincristine and thalidomide or to the disease itself. If caught early, peripheral neuropathy can be effectively managed with dose reduction and is generally reversible.

(ii) If you think that the new technology has any **disadvantages** for patients compared to current standard practice, please describe. Disadvantages might include:

- inconvenience of use (for example is it a treatment that has to be given by somebody else or in hospital?)

As with the majority of cancer / myeloma treatment, Velcade needs to be administered by somebody else and in the hospital setting. This does not pose a problem for the vast majority of patients.

You should remember that patients who require Velcade are at a difficult time in their life and the vast majority would go to the most incredible lengths to get this or any other treatment that offered hope and extra life.

A twice weekly visit to the hospital for a few hours, while for some is a considerable effort, is one that patients are only too happy to make.

- more side effects (for example number of problems, how often, for how long, how severe).

Most patients describe the side-effects on Velcade as being fairly tolerable and no worse than those experienced with any other treatment they have received for their myeloma.

The most trouble symptom is peripheral neuropathy but once gained, this is well tolerated in the vast majority of patients and is commonly reversible if managed correctly.

If you are familiar with the evidence base for the technology, please comment on whether patients' experience of using the technology as part of their routine NHS care reflects that observed under clinical trial conditions.

I am aware of the evidence base and in my experience the clinical benefits I have seen in patients the length and breadth of the country are on par with published data.

Many patients describe a very noticeable improvement in quality of life even although they may have only partially responded to the treatment.

What is without doubt though is the real hope that this treatment gives patients to keep going and surviving longer is not quantifiable in any clinical study. Without hope, we may as well pack our bags and give up.

Are there any adverse effects that were not apparent in the clinical trials but have come to light since, during routine NHS care?

Without being controversial, the most common adverse effect seen now that was not obvious in the trials has been the inability for patients who need this treatment to gain access.

This has caused more harm, physical and emotional pain, disease deterioration, anger and frustration than any actual adverse effect from the treatment itself. The system is the adverse effect.

I very, very, very much welcome this STA and we must approve this and other potential life saving cancer drugs as quickly as possible. It is absolutely nothing short of criminal not to do.

Any additional sources of evidence?

Are you aware of any research carried out on patient or carer views of the condition or existing treatments that is relevant to an appraisal of this technology? If yes, please provide references to the relevant studies [or, please attach copies of the study report]

Implementation issues

Is the following an implementation issue or should we change heading?

What key differences, if any, would it make to patients and/or carers if this technology was made available on the NHS?

I strongly believe that the potential impact upon the lives of patients, cares and their families is very significant and wide ranging and provides a compelling case for a positive approval.

What implications would it have for patients and/or carers if the technology was **not** made available to patients on the NHS?

A negative appraisal would be a devastating blow to patients and the wider myeloma community. I couldn't believe that with such compelling evidence and the pivotal role that Velcade will play in turning myeloma into a cancer that people can live with rather than die from, that the appraisal could be anything other than positive.

Are there groups of patients that have difficulties using the technology?

Only those that cannot get access to the treatment because of postcode prescribing, lack of implementation of government guidance, lack of funding and lack of NICE approval and those that cannot currently afford to pay for it themselves.