

To:

Dr Tomas Salmonson
Chair of the CHMP

Dr Pierre Demolis
Vice-Chair of the CHMP

Brussels, 11 August 2016

Dear Dr Salmonson

Dear Dr Pierre Demolis

Letter to the Chairs of the CHMP on European patient, carer and clinician support for a positive recommendation on ixazomib

We are writing to inform you about the European patient, carer and clinician perspective on the benefit of ixazomib. Whilst we understand the very difficult job that the CHMP has, and we are very supportive of the work you do in assessing new medicines, in the case of ixazomib for relapsed myeloma we do not think that the right decision has been reached.

We consider ixazomib to be a critical new drug in the treatment of relapsed myeloma and one that will significantly add to the range of treatment options available to myeloma doctors and their patients. There is strong clinical support for using ixazomib in relapsed myeloma, as evidenced by the widespread uptake of ixazomib through the EMA approved compassionate use programme.

In particular, we encourage the CHMP to further consider the following information about ixazomib in combination with lenalidomide and dexamethasone:

- It is a safe and effective treatment option for relapsed myeloma patients. Patients and carers also tell us that the side-effect profile for the treatment is tolerable and acceptable to them
- It improves progression free survival (PFS) on average of 6 months compared to lenalidomide and dexamethasone alone. In some groups of patients, including third line and high-risk patients, the PFS was longer than this. This represents a significant and prolonged period for patients and their carers and family members. In interviews with myeloma patients, conducted by Myeloma UK, patients commented:
 - *“The most important factor regarding ixazomib is the increased survival benefit.”*
 - *“When I first relapsed 17 years ago there were very few treatments available. A treatment like ixazomib can be built upon. The additional progression free survival time this treatment gives you could lead to another treatment which could give you years more.”*
 - *“Not making this treatment available would be a huge backward step. Even taking the side-effects into account, I absolutely would take this treatment.”*
- Given the relapsing and remitting nature of myeloma, any PFS gains in myeloma should not be seen in isolation or underestimated. Treatments and survival gains (both PFS and overall survival) should be seen as a “bridge” to subsequent treatment options which may prolong survival even further

- The impact that ixazomib has on PFS is likely to be more substantial when used in the real-world setting, given the increased ability to provide personalised treatment (i.e. through dose modifications, supportive care etc).
- European doctors who have accessed ixazomib via the ixazomib named patient programme have seen patients respond very well to it in the real-world setting and would value the ability to use it routinely in their patients
- Trials involving ixazomib have demonstrated that it works well in patients who have high-risk myeloma and has the potential to overcome the high-risk features of myeloma. This is a major area of unmet need, given that these patients currently have very poor outcomes
- It is the first and currently the only oral proteasome inhibitor. Whilst myeloma patients are largely split between their preferences for oral vs. IV treatment options, it is important that oral treatment options are available for them as it can help patients feel more “in control” of their treatment and also to carry on with their everyday lives without having to attend regular hospital appointments. This is particularly important for patients who find it difficult to attend hospital appointments (i.e. if they are older/frailer or live far away from hospital)
- Given the relapsing and remitting nature of myeloma, it is essential for patients to access a range of novel treatments and treatment combinations at each stage of their disease pathway. Allowing doctors a choice of treatment options and the ability to personalise this to the treatment needs and circumstances of the patient is imperative

On behalf of myeloma doctors, patients and carers, we very much hope that the CHMP overturns its negative recommendation and ensures ixazomib is added to the treatment options for patients. Only through ensuring the approval of new and promising treatments for myeloma patients across Europe, can we keep survival rates in myeloma on an upward trajectory.

Should you have any questions or wish to receive further information, please get in touch with us at policy@mpeurope.org or by calling +44 (0) 131 557 3332.

Yours sincerely



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