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Paul Adrian Harford
[redacted]

Dear Jeremy,

Appraisal Consultation Document – azacitidine for the treatment of Myelodysplastic Syndromes, Chronic Myelomonocytic Leukaemia and Acute Myeloid Leukaemia.

I am naturally disappointed to learn from the ACD the committee's preliminary decision is not to recommend azacitidine for the treatment of the conditions above. However, I do gain some comfort that those of us currently receiving azacitidine can continue to do so.

Comments on the ACD

Sections 2.3 & 3.8
The appraisal has been made on the basis of azacitidine being injected subcutaneously daily for 7 days, followed by a rest period of 21 days.
The dosage is 75mg/m2 involving 9 vials for one cycle. In my treatment I receive an equal dosage of in total 750mg over 5 days which I calculate to be 7.5 vials.

Bearing in mind weekend treatment is not required and what appears to be a lower dosage, I ask whether the cost effectiveness is improved to a more reasonable and acceptable level?

Section 4.5
It is disappointing that no quality of life data were collected in the AZA-001 trial. However, patient experts did confirm the improvement in health and increased ability to perform normal activities of daily living.

I have already confirmed my improved health from my azacitidine treatment which I have received form April 2008. The improvement was a quick one in that no blood transfusions have been necessary since July of 2008.
A most important feature of this has been a consistent haemoglobin and platelet level within the normal parameters.
The problem with blood transfusions was the decline in haemoglobin level in the period between each transfusion which were necessary every 3 weeks involving either 2 or 3 units.
Best Supportive Care treatment only therefore offered a temporary respite compared with no decline with treatment with azacitidine.

What has not been mentioned with care by blood transfusions is the increasing risk of iron (ferritin) content in the blood.

One other important factor is the risk of infection on Best Supportive Care because of a breakdown in one's immune system. I did experience an infection as result.
I had to be hospitalized for 6 days, after which I was treated for a period of 14 days with intravenous anti-biotics.

I hope my comments and observations will be of use in reaching the final recommendations.

Yours sincerely,

Paul A. Harford
(Patient Expert – azacitidine HTA)
2 August 2009