THE LEUKAEMIA SOCIETY (UK)
MDS UK Patient Support Group
RARERcancers FORUM

25th May 2010

By First Class Post and Email

[redacted]
National Institute for Health and Clinical Excellence
MidCity Place
71 High Holborn
London
WC1V 6NA

Dear [redacted]

Response to Appeal Panel's Legal Advice on Human Rights

Thank you for providing us with the legal advice to the Appeal Panel on the application of the European Convention of Human Rights ("ECHR") to this appeal and the opportunity to respond to this advice. We have had sight of Celgene's note of submissions on human rights and we agree entirely with those submissions. However, we wish to elaborate on what the fundamental human rights at issue mean to patients.

Article 2: The Right to Life

Azacitidine offers patients with higher risk MDS a life line of nearly 10 months when compared with best supportive care and nine months on average overall. However, these figures are just averages. Many patients live much longer than this and reports of patients receiving azacitidine and living for more than two years at a high quality of life are not uncommon. This length of time is considerable and allows patients to spend longer with their families, continue to work and keep active and ultimately have hope for the future in light of the enormous amount of new drug development research being conducted in MDS.

To this end, we disagree with [redacted] statement at paragraph 2 of his advice where he states that "it is common ground that the effect of the drug is to delay death from the illness treated, rather than to cure the illness outright". This is not common ground as there are patients who were deemed ineligible for stem cell transplantations but are now considered eligible after receiving azacitidine.

As we make clear in ground 1 of our appeal, NICE chose to ignore data on quality of life offered to it by MDS Patient UK and therefore does not appear to have understood the quality of life experienced by patients taking azacitidine and therefore the importance of securing our right to life under Article 2 of the ECHR.

Finally, we believe that our argument under Article 2 is supported by the fact that doctor's are ethically obliged to take action to prolong life if the treatment is not considered to be excessively burdensome or disproportionate in relation to the expected benefits. This obligation has recently been developed further and the GMC has since issued specific guidance on end-of-life therapies that states that doctors must start from a presumption in favour of prolonging life. This presumption will require doctors to take all reasonable steps to prolong a patient's life. Telling patients that they cannot receive azacitidine and must instead receive best supportive care and/or chemotherapy is unethical as it effectively robs the patient of the right to a significant and high quality life extension.

Article 3: Inhuman or degrading treatment

Although we recognise that the threshold for engaging this Article is high in circumstances where there is no "deliberate" infliction of pain or suffering, we believe that the threshold is met given that patients with high risk MDS are in a life-threatening and highly debilitating situation and azacitidine would afford them a considerable and enhanced life-extension. We firmly believe, therefore, that denying patients azacitidine is tantamount to an unnecessary and premature sentence to death under debilitating conditions that can only be described as inhuman and/or degrading.

In addition, compared with azacitidine, best supportive care is inhuman and/or degrading as it involves repeated blood transfusions that take a physical toll on the body, particularly for those who find it difficult to tolerate blood transfusions. The time involved in travelling to the transfusion centre, receiving the transfusions, and the necessity of having a caregiver accompany the patient imposes a hardship on patients' lives and those of their caregivers. With repeated transfusions, the burden becomes higher as the disease progresses as does the risk of end organ complications arising from iron overload. In contrast, patients receiving azacitidine have significantly fewer blood transfusions and in some cases are transfusion independent. Similarly, chemotherapy has a much more toxic profile compared with azacitidine and some patients are unable to tolerate it.

Article 8: Right to Respect for Private and Family Life

The negative recommendation infringes MDS patients' rights under Article 8 to the private and family life that an overall 9 months of life extension would provide, particularly as the Appraisal Committee has failed to give due or any regard to the wishes and fears of MDS patients as demonstrated by NICE ignoring MDS UK's data on MDS and quality of life.

Many patients with MDS have families, children and grandchildren. The extra time that patients who receive azacitidine can spend with their families is therefore extremely precious. In addition, patients eligible for azacitidine can now carry out a wider range of day-to-day tasks and enjoy more autonomy and self-determination. One of the patient experts, [redacted], gave evidence to the Appraisal Committee saying that since taking azacitidine he has been able to go to football games, take up gardening, socialise and go shopping. He also believed that had he been on azacitidine he would have been able to continue working whereas before he was simply too tired.

Article 14: Age Discrimination

Whilst we accept that the FAD does not directly discriminate between old and younger patients with MDS in terms of access to azacitidine it does generate issues of equality in terms of access to effective therapy. Younger patients (a minority of cases) can be effectively treated with intensive chemotherapy followed by an allogeneic stem cell transplant. Denying this young minority of patients access to azacitidine does not necessarily, therefore, deny them effective therapy. However, for the elderly majority, intensive chemotherapy and allogeneic stem cell transplantation are not always an option because of toxicity. Consequently, to deny them azacitidine will be denying them the single most effective and currently applicable therapy therefore indirectly discriminates against elderly people.

We remain available for any further assistance that we can give you.

Yours sincerely

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Chairman, MDS UK Patient Support Group

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[redacted]
Deputy Chairman, MDS UK Patient Support Group