United Parent Projects
Muscular Dystrophy

*

Nicolas Rossignol
European Commission
Pharmaceuticals Unit F/2
Directorate-General Enterprise and Industry
Office 10/128, Breydel Building
Avenue d'Auderghem 45
B-1040-Brussels
België

Subject: PUBLIC CONSULTATION PAPER - PROPOSALS TO AMEND ANNEX I TO DIRECTIVE 2001/83/EC AS REGARDS ADVANCED THERAPY MEDICINAL PRODUCTS

Amsterdam, June 14th 2008,

Dear Mr Rossignol,

On behalf of the United Parent Projects Muscular Dystrophy, the global organisation of parents of children with Duchenne Muscular Dystrophy, the most common fatal genetic disorder diagnosed in childhood, affecting approximately 1 in every 3,500 live male births, I would like to comment on the above mentioned paper.

We are concerned that the proposal to redefine gene therapy to include antisense drugs that induce exon skipping will cause delays in clinical trials, and ultimately the approval and use of important new therapies for patients that severely need them.

We are committed to finding a treatment for our sons. It goes without saying that we are concerned about the safety of new therapeutic agents being used on our sons and are opposed to any therapies that risk their safety. However, this redefinition is inappropriate and misleading. As advocacy group, we are keenly aware of the differences between therapeutic drugs like these for exon skipping versus biological agents that introduce new genes in viruses. It is surprising to us that there is confusion about this within the drug regulatory community.

We understand it is misleading because of 2 major differences between traditional gene therapy and antisense drugs which will really make them fundamentally different when it comes to their safety profile.
1) Antisense drugs can be stopped immediately and their effects will reverse, versus viral vectors which are 'forever'
2) Antisense drugs don't replace/change or manipulate the gene, only the gene product.

We are concerned that mislabelling antisense drugs as gene therapy might needlessly scare away the very people who most need the drugs. We share your concern about gene therapy and are all too familiar with the well publicized failures of virally-mediated gene therapy. For that very reason non-genetic approaches to treating this disease are our best hope at the moment. To have those drugs misclassified might mean a longer path to getting a real treatment to our sons.

Thanks to my participation in several ethics committees , including the ethics committee of Treat-NMD (an EU finding network of excellence), I know the "reversible" character of antisense therapy versus the more permanent gene therapy should lead to different judgements from an ethics committee when it comes to clinical trial applications. It would be problematic for ethics committees if these two very different therapies with very different risk profiles were confused. This amendment has the potential to lead to such confusion and only prolong the process of getting this potential therapy into the clinic.

As the advocacy group for these boys, we are compelled to comment when we feel that unnecessary obstacles may impede the fight against this disease. This amendment does not further the cause of the patients with this disease and will slow the progress of one of the treatment modalities that has shown activity (van Deutekom NEJM 2007 357:2677-2686).

Please keep in mind we are looking at a population of young men and boys with a progressive disabling and fatal disorder. Time matters!

Last but not least, as you can see in the above mentioned paper, patient organisations have been the major sponsors of the research leading to this potential treatment. Parents, who have spent a lot of time and energy raising money while having sick children at home, do not want to see those efforts squandered due to inappropriate red-tape because of this mislabelling.

We hope that the Commission understands how desperately these patients need new treatments, and we request that if there is a forum for discussing this amendment that our group is invited to participate. We are actively seeking to prevent this error from being codified.

Yours sincerely,

Drs Elizabeth Vroom
Chair, United Parent Projects MD
Postbus 821, 3900AV Veenendaal
The Netherlands
Chamber of Commerce nr UPPMD 30226334
Tel nr +31 164 230270
www.duchenne-community.com

Attached: Paper van Deutekom NEJM 2007 357:2677-2686