PUBLIC CONSULTATION PAPER ON THE REGULATION ON ADVACED THERAPY MEDICINAL PRODUCTS

Comments by

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General remarks

During the EATB Congress which took place 21. -23. November 2012 in Vienna I had the honor to chair a session corncerning advanced therapies and hospital exemption.

There was a lively discussion about the hospital exemption in particular and the ATMP regulation in general.
Participants from all over europe came to a general consensus which I woudl like to present.

2.1 Marketing Authorisation

Advanced therapies are very different from classical pharmaceutics.

Due to the lobbying the regulation was designed mainly to meet the needs of big pharmaceutical investors. and therefore designed in a very traditional way.

That is why advanced therapy regulation does not really cover the needs of the topic.

For most of the therapies it does not make sense to go for the common market for numberous reasons.
Most of the treatments on the market are for autologous use that means tailor-made therapies for individual patients.
Manufacturers have to stay in a very close relationship to clincs and spezialized medical doctors, so there is a strong need to stay local.
Transport is a critical part and often time dependent.
Optimal timing of the surgical procedures is essential.
It`s very similar to in vitro fertilisation. No one would ever think of going to the EU market with in vitro fertilisation.

EU has encouraged that the topic should be covered by non profit or public health organisations but this organisations are not entiteled to go for the european market resp. to go for profit and big business.
Many of the SME´s in this field have less than 10 employees and a turnover under 500 000€ a year.
Often it is an „orphan drug situation“ with a very small number of patients.

And looking at what these lab`s are doing: they provide a service

There is a remarkable judgement of the European surpreme court (Case C-156/09):
In terms of value added taxes :

„...must be interpreted as meaning that the removal of joint cartilage cells from cartilage material taken from a human being and the subsequent multiplication of those cells for reimplantation for therapeutic purposes constitute 'provision of medical care' in accordance with that provision.“

That means : processing of autologous cells is not classified as „manufacturing of a medical product“ but as „a service providing medical cure“

There is no marketable product in the common sense:
There is no single substance,
no big batch that could be sold to anyone,
manufacturers are not owner of the cells,
they cannot use it except for one specific patient,
what if they would deny the application if the patient is willing to take the chance of a cure (maybe it is the only one)?
The cells are the personal property of the patient and cannot be subject to commercial interests.!

Hence, there is **no market** for such products!
In conclusion there is no real need of a marketing authorisation for a product.

The need would be a harmonised european authorisation for the manufacturing procedure.

2.3 Hospital Exemption

SME-manufacturers are de facto contract manufacturers of a clinic or/and a medicinal practitioner and that is exactly what the hospital exemption is about.

The hospital exemption sees the responsibilities at the right point:
Manufacturers have to take care of quality and savety according to all the regulations given.
Clinics and medical doctors are resposible fort the treatment for it is a medical treatment and there can`t be a autorisation for a medical treatment.

That is exactly what most of the member states have implemented with great success in the last decade and this works remarkably well.

2.2 Combined products
Tissue Engineering is always in, one way or another, involving combined products.
All of these medical devices have already a marketing authorisation.
Application resp. combination sometimes takes place during second surgical procedure.
The cost of such advanced therapy procedures are already very high.
A centralised authorisation causes enormous aditional cost without any benefit for quality or safety.

Due to the low number of applications and the autologous setting these cost cannot, in what way ever, be recoverd - there is no realistic way of return on investment for SME´s or non profit organisations.
That ist the reason why no one applys for a marketing authorisation.

In europe there is no patentability of a medical treatment like advanced tehrapies so the whole thing was handled more like a open source project.. Lots of researchers and lab`s all over europe have contributed to science and developement in this field. Most of the funding comes from european tax payers.

This justifies the more open approach as done by hospital exemption.

There is a strong tendency that marketing authorisation is misused by pharmaceutic industry as a vehicle to install unjustified property rights to monopolise the market and needless to say for cherry picking of profitable applications.

We have to look at patients needs:
Patients want medicinal products which are beneficial, save, available and most of all affordable.
Centralised authorisation done in the traditional pharmaceutical way leads to a cost explosion of five to tenfold of the already expensive procedures with no further benefit for patients!
Costs are not only in the hands of manufacturers but also and mainly in the hands of the regulatory athorities!
Our health systems are at their financial limits so its our responsibility to keep costs down, that should be considered at EMA

2.5. Scope and adaptation to technical progress.
Autologous procedures should be excluded from the scope of the ATMP regulation.
We would reccomend a network of local labs, maybe certified, with high quality standards which can provide all these services conform to adequate GMP requirements and conform to other EU regulations (Directive 2004/23/EC) for maximum safety simultaneously at a reasonable price.

These small entities play a crucial role in science and developement of this new medical treatments which is still a fast ongoing process .
So its the best to have these invaluable, small, local entities which are well known to the local regulatory authorities and together they can handle the fast progress in science in a good and save way which was proven over the last 10 – 15 years.
Everyone involved can gain experience that can eventually lead to a more adequate and harmonised european autorisation procedure for the process of manufacturing.