

## **A position paper from the Genetic Interest Group**

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### **Introduction**

The Genetic Interest Group (GIG) is the UK alliance of patient groups supporting families with all types of genetic disorder. Its membership includes small groups run entirely by voluntary effort, supporting families with very rare conditions arising from mutations in single genes; to large organisations for those with common conditions where genetics plays a part alongside environmental, lifestyle or other factors in precipitating the development of the condition and/or modifying its progress.

All our members support families affected by conditions which are at best intractable. Most are incurable given current knowledge, and all produce chronic ill health, often progressive disability and not infrequently, premature death.

For the families affected by, or at risk of these conditions, high quality biomedical research and the development of innovative therapies created from the new knowledge that such research generates offers hope for the future. For many of those with genetic conditions the therapies they hope for are likely to arise from developments in areas of R & D covered by the proposed regulation on Advanced Therapies / Tissue Engineered Products.

### **The need for regulation**

All patients hope to be able to receive therapies that are safe, effective and of reliably high quality. A clear, robust regulatory framework is an essential component in securing quality, safety and efficacy, and GIG welcomes the proposal to develop a regulatory framework that will cover advanced therapies and tissue engineered products.

The aim of such a regulatory framework must be to promote good science, and to maximise the likelihood of its outputs being translated into products and services that will benefit those with currently unmet medical needs, as well as to protect patients from undue pressure and risk arising from premature exposure to under-developed and potentially unsafe interventions.

Clearly this is an area where absolute certainty is impossible, so regulators must be able to strike a balance between risks and benefits – always remembering that stifling development leaves patients and families living with the consequences of diseases that might otherwise have been become treatable or even curable.

The areas covered by the proposed regulation are fast moving, developments until recently thought to be unlikely or impossible are becoming increasingly possible. For this reason GIG favours an approach to regulation which lays out broad principles and goals, leaving the implementation of the detailed action necessary to achieve these to a properly constituted and empowered statutory body, rather than through detailed prescriptions and proscriptions which may, as our knowledge evolves, turn out to limit steps that are universally seen to be desirable, whilst permitting others unforeseen to slip through loopholes in the regulatory net.

Turning to the Commissions' proposals we offer the following observations and recommendations.

## 1. Definitions and Scope

There would appear to be the potential for confusion as to whether the appropriate route for regulation is by development of procedures appropriate for the licensing of medicinal products or those for medicinal devices, especially where combination products are envisioned. GIG does not have a view as to which route is likely to provide the most effective way of stimulating innovation and/or protecting patients. Where there is the possibility of confusion almost inevitably this creates scope for argument and delay whilst differences are sorted out. This is to the ultimate detriment of the patient waiting to benefit.

**GIG therefore recommends that, whichever regulatory pathway is deemed to be appropriate, the result is the production of sufficient high-quality accessible information about the product and its characteristics to enable patients to make informed decisions as to risks and benefits associated with its use.**

**GIG also recommends that patients should be fully involved in decisions regarding the selection of criteria for the determination of quality, safety and efficacy, in order to ensure that the resulting product is likely to have the greatest possible impact on their unmet medical needs.**

GIG welcomes the recognition of the particular circumstances relating to the small scale manufacture of tissue engineered products in hospital environments. Some hospitals have launched cell or tissue therapy units in which they develop small-scale technologies such as immune cell therapies for cancer patients, nervous tissue transplants for paraplegic patients, and pancreatic islet-cell transplants to combat diabetes; as well as the more established therapies based on bone marrow. If these small research departments were made to follow the same legislative procedures as medium and large scale organisations, research progress would suffer badly.

Therefore, GIG believes that where products are developed by healthcare professionals for the treatment of patients under their care, the requirement to seek a full marketing authorisation would be disproportionate and potentially deleterious to patient wellbeing. Laboratories producing such "one-off" products should be regulated by appropriate professional frameworks (such as NEQAS in the UK) to ensure compliance with Good Laboratory Practice (GLP) standards, **but only if a decision to commercialise such products is taken should they fall within scope of the proposed regulation.**

## 2. Committee for Advanced Therapies

We welcome the proposal to establish a specialist committee for the regulation of advanced therapies and tissue engineered products at EMEA. **Such a committee should be able to pass independent judgements and make recommendations for the granting of marketing authorisations in its own right.** The level of expertise in the fields covered by the proposed regulation is likely to be greater amongst the members of the proposed CAT than on the CHMP, and decisions made are likely to be more rigorous and better founded. Requiring CAT decisions to be ratified by the CHMP is likely to cause unnecessary delays and create duplication to no good purpose.

**GIG strongly endorses the plan for the inclusion of two representatives of patient organisations** in article 21 of chapter 7 of the proposal for the regulation of advanced therapies. The experience of the inclusion of patient members on the COMP (Committee for Orphan

Medicinal Products), and now on the EMEA management board has been entirely positive in our view, and the CAT would derive benefit from the input of patient views to their procedures.

### 3. Impact on Innovation

Given the timescale for innovative therapies to move from “bright idea” to the point at which they can be approved for use in patients, it is important that academics, clinicians and pharma/biotech companies know as soon as possible what regulatory requirements they will be expected to fill. Delay and/or a lack of clarity will result in missed opportunities, a reluctance to invest and gaps in the evidence base necessary for regulatory approval, all of which would be contrary to the best interests of patients.

**GIG recommends that the legislation is enacted and any necessary technical annexes drafted, put out for consultation, modified and implemented as quickly as is consistent with a thorough and careful examination of the relevant issues. Transitional arrangements to cover the overlap between old and new regimes should be clear and announced as soon as possible.**

GIG believes that under the proposed transitional arrangements, allowing two years for currently available therapies and those in the late stage of development to comply with regulatory requirements, companies cannot be certain of their ability to meet the deadline. Whilst quick compliance is desirable, **continuity of the availability of therapy must be the priority when designing interim measures. We recommend measures to ensure useful therapies are not withdrawn from patients in need, due to any delay in compliance.**

### 4. Access for patients

Innovative products are of little use to patients if their availability is restricted and/or the price is unaffordable to health care systems in member states. The incentives offered under the Orphan Medicinal Products regulations have provided an important stimulus for research and development. Given that it is generally small and medium-sized enterprises in the health care biotechnology sector that are most active in this field, similar incentives – including access to scientific advice protocol assistance and other non-financial support, will provide a significant boost for the development of novel products, and hence of health gains to patients.

**GIG recommends that consideration should be given to developing appropriate incentives to encourage innovation to enter this field, with expert advice and support on fulfilling regulatory requirements freely available in a timely and accessible manner.**

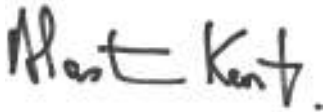
**GIG further recommends that a coordinated mechanism for determining issues of pricing, reimbursement and patient access be developed to ensure speedy and equitable introduction of advanced and tissue engineered therapies across the EU.** This will boost equity for patients and provide a valuable contribution to the EU's knowledge based economy.

### 5. Exclusions & Ethical Issues

Tissue engineering touches on potentially controversial areas from time to time. The use of embryonic stem cells is one such aspect where member states differ in the ethical and legal frameworks adopted.

GIG agrees with the proposal to require long-term patient follow-up and post-authorisation monitoring for those therapies that remain in a patients system longer than conventional therapies.

**GIG welcomes the decision to abide by the principle of subsidiarity, and to exclude the use of embryos and embryonic stem cells for the scope of this legislation. GIG also believes that member states should undergo a public review of the pros and cons of research and development in potentially rewarding but possibly ethically challenging areas in order to develop policies and legislation that reflects the views of their citizens.**

A handwritten signature in black ink that reads "Alastair Kent". The signature is written in a cursive, slightly slanted style.

Alastair Kent  
Director  
**Genetic Interest Group**